Filed pursuant to Rule 424(b)(4)
Registration No. 333-225213

PROSPECTUS

5,247,958 Shares

Common Stock

We are offering 5,247,958 shares of our common stock. This is our initial public offering and no public market currently exists for our common stock. The initial public offering price for our common stock is $19.00 per share. We have been approved to list our common stock on The Nasdaq Global Select Market under the symbol "AVRO."

We are an "emerging growth company" as defined in Section 2(a) of the Securities Act of 1933 and will be subject to reduced public company reporting requirements. See "Prospectus Summary—Implications of Being an Emerging Growth Company."

Investing in our common stock involves a high degree of risk. Please read "Risk Factors" beginning on page 11 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

	PER SHARE	TOTAL
Public Offering Price	*$19.00*	*$99,711,202*
Underwriting Discounts and Commissions (1)	*1.33*	*6,979,784*
Proceeds to us, before expenses	*17.67*	*92,731,418*

(1) See "Underwriters" in this prospectus for a description of compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase an additional 787,193 shares of our common stock. If the underwriters exercise the option in full, the total underwriting discounts and commissions payable by us will be $8,026,751, and the total proceeds to us, before expenses, will be $106,641,118.

Certain of our existing stockholders, including certain affiliates of our directors, have agreed to purchase an aggregate of approximately $23 million of shares of our common stock in this offering at the initial public offering price.

Delivery of the shares of common stock is expected to be made on or about June 25, 2018.

MORGAN STANLEY *COWEN* *WELLS FARGO SECURITIES*

WEDBUSH PACGROW

Prospectus dated June 20, 2018

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Through and including July 15, 2018 (25 days after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the obligation of dealers to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

You should rely only on the information contained in this prospectus or in any free writing prospectus we file with the Securities and Exchange Commission. Neither we nor the underwriters have authorized anyone to provide you with information other than that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are offering to sell, and seeking offers to buy, common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover page of this prospectus, or other earlier date stated in this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock.

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms, or other independent sources that we believe to be reliable sources. We are responsible for all of the disclosure contained in this prospectus, and we believe that these sources are reliable; however, we have not independently verified the information contained in such publications. While we are not aware of any misstatements regarding any third-party information presented in this prospectus, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties, and are subject to change based on various factors, including those discussed under the section titled "Risk Factors" and elsewhere in this prospectus. Some data are also based on our good faith estimates.

PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including our consolidated financial statements and the related notes included elsewhere in this prospectus. You should also consider, among other things, the matters described under "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," in each case appearing elsewhere in this prospectus. As used in this prospectus, unless the context otherwise requires, references to the "company," "we," "us" and "our" refer to AVROBIO, Inc.

Overview

We are a Phase 2 clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral-based gene therapies to treat rare diseases following a single dose. Our gene therapies employ hematopoietic stem cells that are extracted from the patient and then modified with lentiviral vectors to insert a functional copy of the gene that is defective in the target disease. We believe that our approach, which transforms stem cells from patients into therapeutic products, has the potential to provide curative benefit for a range of diseases in an outpatient setting. Our initial focus is on a group of rare genetic diseases referred to as lysosomal storage diseases, which today are primarily managed with enzyme replacement therapies, or ERTs. These lysosomal storage diseases have well understood biologies, identified patient populations and represent large market opportunities with approximately $4.0 billion in worldwide net sales in 2017.

Our initial pipeline is comprised of four lentiviral-based gene therapies, including AVR-RD-01 for the treatment of Fabry disease, AVR-RD-02 for the treatment of Gaucher disease, AVR-RD-03 for the treatment of Pompe disease and AVR-RD-04 for the treatment of cystinosis. AVR-RD-01 is currently being evaluated in an investigator-sponsored Phase 1 clinical trial and our company-sponsored Phase 2 clinical trial to assess safety and toxicity, as well as preliminary efficacy. In addition, Phase 1/2 clinical trials for both AVR-RD-02 and AVR-RD-04 are being planned and we expect patients will be dosed in 2019.

The first two patients in the ongoing Phase 1 clinical trial of AVR-RD-01 have been dosed. The primary goal for this clinical trial is to assess the safety and toxicity of AVR-RD-01. In this trial, safety and toxicity are measured by the frequency of clinically notable abnormal vital signs and laboratory results and the frequency of treatment-related adverse events. A secondary objective for this clinical trial is to obtain preliminary efficacy signals of AVR-RD-01 therapy as assessed by a-galactosidase enzyme activity compared to baseline. Enrollment in this clinical trial is ongoing, with up to six patients with Fabry disease who have been treated with ERT expected to be enrolled. Because this is a Phase 1 trial, the currently approved standard of care, ERT, is suspended for enrolled patients one month prior to receiving AVR-RD-01 and is then resumed one month after AVR-RD-01 treatment. We believe the preliminary results from this trial support the potential of AVR-RD-01 to drive active enzyme production for long durations.

On June 7, 2018, we dosed the first patient in our company-sponsored Phase 2 clinical trial of AVR-RD-01. The primary objective for this clinical trial is to assess safety and efficacy, measured by multiple indicators, such as globotriaosylceramide levels in multiple tissues, organ function, gastrointestinal symptoms and pain and quality of life. Enrollment in this trial is ongoing, with up to 12 treatment-naïve patients with Fabry disease expected to be enrolled.

Lentiviral-based gene therapy has been observed to be well-tolerated in third parties' ongoing clinical trials for rare diseases such as beta thalassemia, ALD and ADA-SCID. To date, over 200 patients have been treated with lentiviral-based gene therapies in third parties' rare disease clinical trials. Historically, the use of *ex vivo* lentiviral-based gene therapies has been restricted primarily to the most acutely severe diseases where risks of the typical requirement for heavily ablating the patients' bone marrow and thus significantly impairing these patients' immune systems had an acceptable risk/benefit profile. The ablation procedure, also known as the conditioning regimen, is administered prior to the gene therapy. The more intensive the conditioning regimen, the greater the risk of toxicity and thus the need for more intensive in-patient monitoring and potential for lengthy hospitalization.

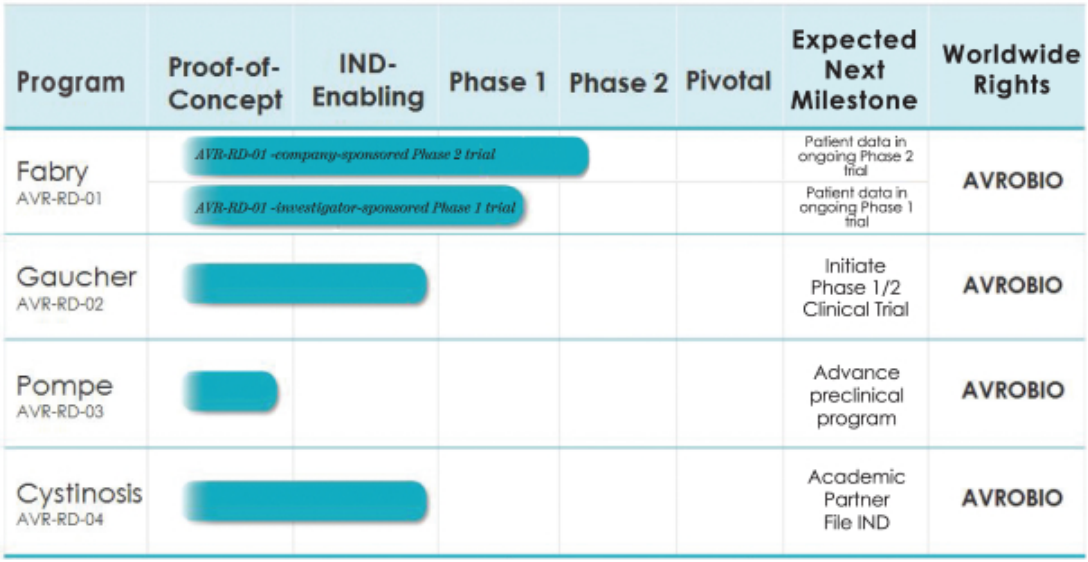
Our goal is to broaden the applicability of lentiviral-based gene therapy by initially targeting diseases where we generally believe durable effects can be achieved following a milder conditioning regimen that allows for outpatient treatment. We believe our approach of choosing diseases where the conditioning regimen can be milder, thus improving patient tolerability, will extend the reach of our gene therapies to a broad range of diseases as first-line therapies.

We are initially targeting rare diseases in which current standard of care provides the mechanistic proof that the enzymes or other proteins produced endogenously following treatment with our gene therapies can offer benefit to patients. Typically in lysosomal storage diseases, a gene mutation results in the deficiency or malfunction of an enzyme or other protein. This results in the inability of lysosomes to properly process cellular byproducts. As a result, these byproducts accumulate to toxic levels in the body's cells and, in turn, disrupt the function of multiple tissues and organs. Fabry disease, Gaucher disease and Pompe disease are primarily managed by bi-weekly, multi-hour infusions with ERTs that seek to exogenously replace the missing enzyme. However, given the characteristics of most ERTs, they typically only remain in the plasma for a short period of time and thus are not ideal because they are only dosed every two weeks. These existing therapies manage, rather than cure, the underlying diseases and, as a result, the diseases continue to progress. Further, the frequent, periodic and life-long dosing schedule required for ERTs results in significant costs for the healthcare system and is burdensome for the patient.

We believe our gene therapies leverage the well understood mechanism of ERTs by transforming a patient's own cells into a drug product that enables the patient to express functional enzyme or other protein and mirror the biology seen in an otherwise healthy individual. We believe that a single dose of our gene therapies may provide meaningful life-long benefit to these patients and potentially cure these diseases while also providing significant health economic advantages.

Our Pipeline

Our programs leverage years of extensive preclinical and early clinical research by leading researchers, as well as our internal research efforts. The status of our lentiviral-based gene therapy programs is reflected below.



AVR-RD-01. Our lead product candidate, AVR-RD-01 for the treatment of Fabry disease, is derived from hematopoietic stem cells to which the gene encoding the enzyme a-galactosidase A, or AGA, is added in an *ex vivo* process using a lentiviral vector. In an ongoing Phase 1 clinical trial of patients with Fabry disease, AVR-RD-01 has led to the production of active AGA enzyme in the two patients treated to date. In both patients, within days of receiving AVR-RD-01, the level of AGA enzyme activity began to rise from nearly undetectable levels before treatment to levels above the range for males with classic Fabry disease. As of twelve months after receiving AVR-RD-01, the first patient's plasma AGA enzyme activity levels continued to be above the range for males with classic Fabry disease. Plasma AGA enzyme activity levels in the second patient remained above the range for males with classic Fabry disease, defined as less than 1 nmol/hr/ml, as of three months after treatment.

Plasma AGA Activity (nmol/hr/ml) Following Treatment with AVR-RD-01

	Day 0 (Infusion of AVR-RD-01)	3 Months	12 Months
Patient 1	0.1	5.8	5.8
Patient 2	0.2	7.6	N/A

We believe these preliminary results support the potential of AVR-RD-01 to drive active enzyme production for long durations. On June 7, 2018, we dosed the first patient in our company-sponsored Phase 2 clinical trial.

AVR-RD-02. We are developing AVR-RD-02 for the treatment of Type 1 Gaucher disease. We will manufacture AVR-RD-02 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that encodes for glucocerebrosidase, or GCase, and then infused into the patient. We plan to initiate a Phase 1/2 clinical trial for AVR-RD-02 in patients with Type 1 Gaucher disease and expect to dose the first patient in this clinical trial in 2019.

AVR-RD-03. We are developing AVR-RD-03 for the treatment of Pompe disease. We will manufacture AVR-RD-03 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that encodes for acid alpha glucosidase A, or GAA, attached to a peptide sequence known as a glycosylation-independent lysosomal targeting, or GILT, tag and then infused into the patient. AVR-RD-03 will incorporate a GILT tag because the addition of a GILT tag has been shown to increase the uptake of GAA into cells, especially in muscle cells, which is a particularly important target tissue for patients with Pompe disease.

AVR-RD-04. We are developing AVR-RD-04 for the treatment of patients with cystinosis. We will manufacture AVR-RD-04 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that encodes for cystinosin, and then infused into the patient. In a planned academic sponsored Phase 1/2 clinical trial, we expect the first patient will be dosed in 2019.

We continue to seek opportunities to expand our approach to other rare and non-rare diseases. We plan to identify and develop future product candidates through our own internal research efforts as well as through collaborations with leading researchers worldwide.

We have developed a detailed plan for the more cost efficient and scalable manufacturing of our product candidates. We are establishing global manufacturing capabilities to support all aspects of the development and, if approved, the eventual commercialization of our gene therapies, from lentiviral vector production to cell processing. We are currently executing on our plans to move to a closed, automated manufacturing system. We also utilize a cryopreservation process that we believe will allow for the global distribution and, if approved, commercialization of our gene therapies.

Our Expertise

We are led by biopharmaceutical experts with extensive experience in gene and cellular therapy and rare diseases. Our team has broad expertise in the clinical, regulatory and commercialization aspects of rare diseases as well as process development and manufacturing for cellular therapies. Members of our management team have held senior positions at Shire, Genzyme, Novartis, Lonza and other companies pursuing development, manufacturing and commercialization of gene and cellular therapies and therapies to treat rare diseases.

Our Strategy

Our goal is to develop and commercialize potentially curative lentiviral-based gene therapies for patients and expand the use of this approach to treat a number of diseases. Key elements of our strategy to achieve our goal include:

- Rapidly advance our initial gene therapies;
- Develop first-line gene therapies for lysosomal storage diseases;
- Globally develop, manufacture and commercialize our gene therapies;
- Industrialize lentiviral-based gene therapy; and
- Leverage our approach beyond our initial indications.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- We have incurred net losses since inception. We expect to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- Our lentiviral-based gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and of subsequently obtaining regulatory approval.
- Our product candidates and the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- AVR-RD-01 is being investigated in an investigator-sponsored ongoing Phase 1 clinical trial, in which two patients have been dosed to date and a company-sponsored Phase 2 clinical trial, in which one patient has been dosed to date. We have not commenced clinical trials for any of our other product candidates. We have never completed pivotal clinical trials, and may be unable to do so for any product candidates we may develop, including AVR-RD-01.
- We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of our product candidates.
- While we intend to seek designations for our product candidates with the FDA and comparable foreign regulatory authorities that are intended to confer benefits such as a faster development process or an accelerated regulatory pathway, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

- We expect to rely on third parties to conduct some or all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.
- Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs or otherwise adversely affect our business.
- Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.
- Our rights to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others. In particular, we have in-licensed certain intellectual property rights and know-how relevant to AVR-RD-01 for our Fabry program and AVR-RD-02 for our Gaucher program, but do not own or license any patents or patent applications covering these product candidates.
- We are aware of issued patents in the United States that cover the lentiviral vectors used in the manufacture of our product candidates. While we believe that we have reasonable defenses against a claim of infringement, there can be no assurance that we will prevail in any such action by the holder of these patents.
- We and our independent registered public accounting firm have identified material weaknesses in our internal control over financial reporting which will require remediation.

Corporate History

We were formed as a corporation under the laws of the State of Delaware in November 2015 under the name AvroBio, Inc. Our corporate name was changed to AVROBIO, Inc. in June 2017. Our executive offices are located at One Kendall Square, Building 300, Suite 201, Cambridge, MA 02139 and our telephone number is (617) 914-8420. Our website address is www.avrobio.com. We do not incorporate the information on or accessible through our website into this prospectus, and you should not consider any information on, or that can be accessed through, our website as part of this prospectus.

The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and ™, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Implications of Being an Emerging Growth Company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, as amended. As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- Only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;
- Reduced disclosure about our executive compensation arrangements;
- No advisory votes on executive compensation or golden parachute arrangements;
- Exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting; and

- An exemption from new or revised financial accounting standards until they would apply to private companies and from compliance with any new requirements adopted by the Public Company Accounting Oversight Board requiring mandatory audit firm rotation.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or SEC. We may choose to take advantage of some but not all of these exemptions. We have taken advantage of reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. We have elected to avail ourselves of the exemption for the delayed adoption of certain accounting standards and, therefore, will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

THE OFFERING

Common stock offered by us	5,247,958 shares
Common stock to be outstanding immediately after this offering	23,149,645 shares (23,936,838 shares if the underwriters exercise their option to purchase additional shares in full)
Underwriters' option to purchase additional shares	We have granted a 30-day option to the underwriters to purchase up to an aggregate of 787,193 additional shares of common stock from us at the public offering price, less underwriting discounts and commissions on the same terms as set forth in this prospectus.
Use of proceeds	We estimate that we will receive net proceeds from the sale of shares of our common stock in this offering of approximately $90.5 million, or $104.4 million if the underwriters exercise their option to purchase additional shares in full, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, to advance our lead product candidate AVR-RD-01; to advance our other product candidates and programs; for our external and internal manufacturing and process development activities; for research and development activities that relate to all of our clinical and preclinical activities; and the remainder for planned general and administrative expenses, the costs of operating as a public company, working capital and other general corporate purposes. For a more complete description of our intended use of the proceeds from this offering, see "Use of Proceeds."
Risk factors	You should carefully read the "Risk Factors" section of this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.
Nasdaq Global Select Market symbol	"AVRO"

Certain of our existing stockholders, including certain affiliates of our directors, have agreed to purchase an aggregate of approximately $23 million of shares of our common stock in this offering at the initial public offering price.

The number of shares of our common stock to be outstanding after this offering is based on 2,581,474 shares of our common stock outstanding as of March 31, 2018, and gives effect to the conversion of all of our outstanding preferred stock into 15,320,213 shares of our common stock immediately prior to the closing of this offering, and excludes:

- 1,788,750 shares of common stock issuable upon the exercise of stock options outstanding as of March 31, 2018, with a weighted-average exercise price of $2.56 per share;
- 6,850 shares of common stock issuable upon the exercise of a warrant to purchase preferred stock outstanding as of March 31, 2018 that will automatically become a warrant to purchase common stock upon the completion of this offering, with an exercise price of $3.2845 per share;
- an additional 58,472 shares of common stock reserved for issuance under our Amended and Restated 2015 Stock Option and Grant Plan as of March 31, 2018, which shares will no longer be reserved following this offering;
- an additional 616,300 shares of common stock that were made available for future issuance under our 2018 Stock Option and Grant Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- an additional 223,200 shares of common stock that were made available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

Unless otherwise indicated, all information in this prospectus reflects or assumes the following:

- the filing of our amended and restated certificate of incorporation and the effectiveness of our amended and restated by-laws upon the closing of this offering;
- the automatic conversion of all of our outstanding shares of preferred stock into an aggregate of 15,320,213 shares of common stock upon the closing of this offering;
- no issuance or exercise of outstanding options or warrants after March 31, 2018;
- a 1-for-4.132 reverse split of our common stock effected on June 7, 2018; and
- no exercise by the underwriters of their option to purchase up to 787,193 additional shares of common stock in this offering.

SUMMARY CONSOLIDATED FINANCIAL DATA

The summary consolidated statements of operations data presented below for the years ended December 31, 2016 and 2017 are derived from our audited consolidated financial statements included elsewhere in this prospectus. The consolidated statements of operations data for the three months ended March 31, 2017 and 2018 and the consolidated balance sheet data as of March 31, 2018 have been derived from our unaudited condensed consolidated financial statements included elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information contained in those statements. Our historical results are not necessarily indicative of the results that may be expected in any future period. You should read the following summary consolidated financial data together with the information in the sections titled "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this prospectus.

	Year Ended December 31,		Three Months Ended March 31,	
	2016	2017	2017	2018
	(in thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Operating expenses:				
Research and development	$ 2,663	$ 15,191	$ 1,434	$ 5,647
General and administrative	1,962	3,195	610	2,141
Total operating expenses	4,625	18,386	2,044	7,788
Loss from operations	(4,625)	(18,386)	(2,044)	(7,788)
Other income (expense):				
Interest income	6	57	4	158
Change in fair value of preferred stock warrant liability	—	(17)	—	(12)
Change in fair value of derivative liability	(39)	(283)	(32)	(587)
Other expense	(6)	(19)	(5)	(13)
Total other expense, net	(39)	(262)	(33)	(454)
Net loss	$ (4,664)	$ (18,648)	$ (2,077)	$ (8,242)
Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	$ (4,664)	$ (18,648)	$ (2,077)	$ (8,242)
Accretion of redeemable convertible preferred stock to redemption value	(305)	(85)	(47)	(2,243)
Net loss attributable to common stockholders	$ (4,969)	$ (18,733)	$ (2,124)	$ (10,485)
Net loss per share attributable to common stockholders—basic and diluted(1)	$ (2.44)	$ (8.38)	$ (0.97)	$ (4.51)
Weighted-average common shares outstanding—basic and diluted(1)	2,038,025	2,235,865	2,181,715	2,324,790
Pro forma net loss per share attributable to common stockholders—basic and diluted(1)		$ (2.69)		$ (0.51)
Pro forma weighted-average common shares outstanding—basic and diluted(1)		6,922,173		16,187,901

(1) See Notes 2 and 13 to our audited consolidated financial statements and Note 12 to our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus for further details on the calculation of basic and diluted net loss per share attributable to common stockholders and on the calculation of pro forma basic and diluted net loss per share attributable to common stockholders.

	As of March 31, 2018		
	Actual	Pro Forma(2)	Pro Forma As Adjusted(3)
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 57,928	$ 57,928	$ 146,438
Working capital(1)	53,420	53,420	142,431
Total assets	60,216	60,216	148,170
Warrant to purchase redeemable convertible preferred stock	47	—	—
Derivative liability	958	958	—
Redeemable convertible preferred stock	87,500	—	—
Total stockholders' (deficit) equity	(33,511)	54,036	143,449

(1) We define working capital as current assets less current liabilities.

(2) The pro forma balance sheet data gives effect to (i) the conversion of all outstanding shares of redeemable convertible preferred stock into an aggregate of 15,320,213 shares of common stock upon the closing of this offering; and (ii) the outstanding warrant to purchase shares of our redeemable convertible preferred stock becoming a warrant to purchase shares of our common stock upon the closing of this offering.

(3) The pro forma as adjusted balance sheet data gives effect to the pro forma adjustments described in footnote (2) above, as well as (i) our issuance and sale of 5,247,958 shares of common stock in this offering at the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us and (ii) the payment by us of an aggregate of $2.0 million to University Health Network pursuant to the Stock Purchase Agreement, dated as of January 27, 2016, between us and University Health Network, in connection with this offering. Additionally, for purposes of the pro forma as adjusted amounts shown above, the net proceeds to be received by us from the sale of common stock in this offering have been increased by approximately $55,000 to reflect the estimated offering expenses that had been paid by us as of March 31, 2018.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all other information in this prospectus, including our consolidated financial statements and related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before investing in our common stock. Any of the risk factors we describe below could adversely affect our business, financial condition or results of operations. The market price of our common stock could decline if one or more of these risks or uncertainties were to occur, which may cause you to lose all or part of the money you paid to buy our common stock. Additional risks that are currently unknown to us or that we currently believe to be immaterial may also impair our business. Certain statements below are forward-looking statements. See "Special Note Regarding Forward-Looking Statements" in this prospectus.

Risks related to our financial position and need for additional capital

We have incurred net losses since inception. We expect to incur net losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred net losses. We incurred net losses of $4.7 million, $18.6 million and $8.2 million for the years ended December 31, 2016 and 2017, and the three months ended March 31, 2018, respectively. We historically have financed our operations primarily through private placements of our preferred stock. We have devoted substantially all of our efforts to research and development, including clinical and preclinical development of our product candidates, as well as assembling our team. We expect that it will be several years, if ever, before we have commercialized any product candidates. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- continue our development of our product candidates, including continuing enrollment in our recently initiated Phase 2 clinical trial for AVR-RD-01;
- initiate additional clinical trials and preclinical studies for our other product candidates;
- seek to identify and develop or in-license additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- seek to industrialize our *ex vivo* lentiviral gene therapy approach into a robust, scalable and, if approved, commercially viable process;
- hire and retain additional personnel, such as clinical, quality control, commercial and scientific personnel;
- expand our infrastructure and facilities to accommodate our growing employee base, including adding equipment and physical infrastructure to support our research and development; and
- transition our organization to being a public company.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we seek to complete preclinical and clinical trials of our product candidates, and manufacture, market and sell these or any future product candidates for which we may obtain marketing approval, if any, and satisfy any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business

or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

We have never generated revenue from product sales and do not expect to do so for the next several years, if ever.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We do not anticipate generating revenues from product sales for the next several years, if ever. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', success in:

- completing research and preclinical and clinical development of our product candidates and identifying new lentiviral-based gene therapy product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualifying for adequate coverage and reimbursement by government and third-party payors for our product candidates;
- establishing and maintaining supply and manufacturing processes and relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for our product candidates, if approved;
- obtaining market acceptance of our product candidates, if approved, as a viable treatment option;
- addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations under such arrangements; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by FDA or other foreign regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Even if this offering is successful, we will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate further clinical trials of and seek marketing approval for, our product candidates and continue to enhance and optimize our vector technology and manufacturing processes. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on reasonable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our other product candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs associated with our manufacturing process development and evaluation of third-party manufacturers
- revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the terms of our current and any future license agreements and collaborations; and
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, will be derived from or based on sales of products that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or cause us to relinquish valuable rights.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity, convertible debt securities or other equity-based derivative securities, your ownership interest will be diluted and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. Any additional indebtedness we incur would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline and existing stockholders may not agree with our financing plans or the terms of such financings. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, or our product candidates, or grant licenses on terms unfavorable to us. Adequate additional financing may not be available to us on acceptable terms, or at all.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a development-stage company founded in November 2015. Our operations to date have been limited to corporate organization, recruiting key personnel, business planning, raising capital, acquiring rights to our technology, identifying potential product candidates, undertaking preclinical studies and planning and supporting

clinical trials of our product candidates and establishing research and development and manufacturing capabilities. Although we recently initiated our Phase 2 clinical trial for AVR-RD-01, we have not yet demonstrated the ability to complete clinical trials of our product candidates, obtain marketing approvals, manufacture products on a clinical or commercial scale or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history. In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

Risks related to the discovery and development of our product candidates

Our lentiviral-based gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and of subsequently obtaining regulatory approval.

We have concentrated our research and development efforts on our lentiviral-based gene therapy approach, and our future success depends on our successful development of viable gene therapy product candidates. There can be no assurance that we will not experience problems or delays in developing new product candidates and that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all. For example, the transition to LV2 or of our cell processing to an industrialized, automated closed system using all disposable supplies may not be successful or may experience unforeseen delays, which may cause shortages or delays in the supply of our products available for clinical trials and future commercial sales, if any. In addition, there is no assurance that products using our proprietary LV2 or manufactured using this automated system will achieve the same favorable preliminary results observed to date in the Phase 1 clinical trial of AVR-RD-01.

In addition, the clinical trial requirements of the FDA and other foreign regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates. To date, only a limited number of gene therapies have received marketing authorization from the FDA or foreign regulatory authorities. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States, Canada or other major markets or how long it will take to commercialize our product candidates, if any are approved. Approvals by foreign regulatory authorities may not be indicative of what the FDA may require for approval, and vice versa.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise the CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health, or NIH, also are potentially subject to review by the NIH Office of Science Policy's Recombinant DNA Advisory Committee, or the RAC, in limited circumstances. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and authorized its initiation. Conversely, the FDA can put an investigational new drug application, or IND, on clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution, to conduct a clinical trial, that institution's institutional biosafety committee, or IBC, as well as its institutional review board, or IRB, would need to review the proposed clinical trial to assess the safety of the trial and may determine that RAC review is needed. In addition, adverse

developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, foreign regulatory authorities may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.

The FDA, NIH and the European Medicines Agency, or EMA, have each expressed interest in further regulating biotechnology, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the federal and state level in the United States, as well as the U.S. congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates.

These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be materially and adversely affected.

The FDA recently announced that it is preparing to release a series of draft guidance regarding potential accelerated approval endpoints for certain gene therapy products and other clinical and manufacturing issues related to gene therapy products. We cannot be certain when such guidance will be issued or whether any such guidance will address accelerated approval endpoints or other clinical or manufacturing issues that will be relevant to or have an impact on our gene therapy candidates or the duration or expense of any applicable regulatory review processes.

Our product candidates and the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

During the conduct of clinical trials, patients may experience changes in their health, including illnesses, injuries, discomforts or a fatal outcome. It is possible that as we test AVR-RD-01 or other product candidates in larger, longer and more extensive clinical programs, or as use of our product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier clinical trials, as well as conditions that did not occur or went undetected in previous clinical trials, will be reported by subjects. Gene therapies are also subject to the potential risk that occurrence of adverse events will be delayed following administration of the gene therapy due to persistent biological activity of the genetic material or other components of the vectors used to carry the genetic material. Many times, side effects are only detectable after investigational products are tested in larger scale, pivotal clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. If additional clinical experience indicates that AVR-RD-01 or any other product candidate has side effects or causes serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked or limited.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other clinical trials. Gene therapy is still a relatively new approach

to disease treatment and additional adverse side effects could develop. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients. Another traditional safety concern for gene therapies using viral vectors has been the possibility of insertional mutagenesis by the vectors, leading to malignant transformation of transduced cells. While our lentiviral gene therapy approach is designed to avoid immunogenicity after administration, there can be no assurance that patients would not create antibodies that may impair treatment. If any of our gene therapy product candidates demonstrates adverse side effects at unacceptable rates or degrees of severity, we may decide or be required to halt or delay clinical development of such product candidates.

In addition to side effects caused by our product candidates, the conditioning, administration process or related procedures also can cause adverse side effects. A gene therapy patient is generally administered cytotoxic drugs to remove stem cells from the bone marrow to create sufficient space in the bone marrow for the modified stem cells to engraft and produce their progeny. This procedure compromises the patient's immune system. While certain of our product candidates are designed to utilize outpatient, milder conditioning regimens that are intended to require only limited removal of a patient's bone marrow cells, our conditioning regimens may not be successful or may nevertheless result in adverse side effects. For example, in the ongoing investigator-sponsored Phase 1 clinical trial and our ongoing company-sponsored Phase 2 clinical trial of AVR-RD-01, several adverse events, including suppression of white blood cell and platelet counts, nausea and/or vomiting following the conditioning process, were observed. If in the future any such adverse events caused by the conditioning process or related procedures continue at unacceptable rates or degrees of severity, the FDA or other foreign regulatory authorities could order us to cease development of, or deny approval of, AVR-RD-01 or our other product candidates for any or all targeted indications. Even if we are able to demonstrate that adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the clinical trial.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients, a communication plan to health care practitioners, and restrictions on how or where the product can be distributed, dispensed or used. Furthermore, if we or others later identify undesirable side effects caused by AVR-RD-01 or our other product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such a product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is distributed, dispensed, or administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations.

AVR-RD-01 is being investigated in an ongoing investigator-sponsored Phase 1 clinical trial and an ongoing company-sponsored Phase 2 clinical trial, and we have not commenced clinical trials for any of our other product candidates. We have never completed a pivotal clinical trial, and may be unable to do so for any product candidates we may develop, including AVR-RD-01.

We are at a very early stage of development for all of our product candidates including AVR-RD-01. As of June 15, 2018, our product candidate AVR-RD-01 has been administered to only two patients in an ongoing

Phase 1 clinical trial and to one patient in our ongoing Phase 2 clinical trial. The ongoing Phase 1 and Phase 2 clinical trials for AVR-RD-01 must be completed, as well as potentially additional pivotal clinical trials in order to obtain FDA approval to market AVR-RD-01. Carrying out later-stage clinical trials is a complicated process. We only recently dosed the first patient in our company-sponsored Phase 2 clinical on June 7, 2018. We have limited experience in preparing, submitting and prosecuting regulatory filings, and have not previously submitted a biologics license application, or BLA, for any product candidate.

In addition, we have not yet conducted clinical trials of any our product candidates in the United States, our interactions with the FDA are expected to be limited for the near future, and we cannot be certain how many additional clinical trials of AVR-RD-01 or any of our other product candidates will be required or how such trials should be designed. In order to commence a clinical trial in the United States, we are required to seek FDA acceptance of an IND for each of our product candidates. We cannot be sure any IND we submit to the FDA, or any similar clinical trial application we submit in other countries, will be accepted. We may also be required to conduct additional preclinical testing prior to filing an IND for any of our product candidates, and the results of any such testing may not be positive. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to a BLA submission and approval of AVR-RD-01 or any of our other product candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, our planned clinical trials, could prevent us from or delay us in commercializing AVR-RD-01.

The ongoing Phase 1 clinical trial of AVR-RD-01 is an investigator-sponsored trial being conducted by University Health Network. In addition, the planned Phase 1/2 clinical trial of AVR-RD-04 will be conducted by our collaborators at the University of California, San Diego. We do not control the design or administration of investigator-sponsored trials, nor the submission or approval of any IND or foreign equivalent required to conduct these trials, and the investigator-sponsored trials could, depending on the actions of such third parties, jeopardize the validity of the clinical data generated, identify significant concerns with respect to our product candidates that could impact our findings or clinical trials, and adversely affect our ability to obtain marketing approval from the FDA or other applicable regulatory authorities. To the extent the results of this or other investigator-sponsored trials are inconsistent with, or different from, the results of our planned company-sponsored trials or raise concerns regarding our product candidates, the FDA or a foreign regulatory authority may question the results of the company-sponsored trial, or subject such results to greater scrutiny than it otherwise would. In these circumstances, the FDA or such foreign regulatory authorities may require us to obtain and submit additional clinical data, which could delay clinical development or marketing approval of our product candidates. In addition, while investigator-sponsored trials could be useful to inform our own clinical development efforts, there is no guarantee that we will be able to use the data from these trials to form the basis for regulatory approval of our product candidates.

Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

Results from preclinical studies or early clinical trials are not necessarily predictive of future clinical trial results and are not necessarily indicative of final results. There is a high failure rate for gene therapy and biologic product candidates proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, the design of a pivotal clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. Our company has limited experience in designing and conducting clinical trials and we may be unable to design and execute a clinical trial to support regulatory approval. To date, we have not received definitive guidance from the FDA or

other foreign regulatory bodies regarding the necessary endpoints for approval of any of our product candidates, including AVR-RD-01. There are no assurances that the FDA or other foreign regulatory bodies will find the efficacy endpoints we propose in future pivotal trials to be sufficiently validated and clinically meaningful, or that our product candidates will achieve the pre-specified endpoints in future pivotal trials to a degree of statistical significance. We also may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Our AVR-RD-02, AVR-RD-03 and AVR-RD-04 product candidates have not yet been tested in humans. Any of our other product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies. Any such failure would cause us to abandon the product candidate.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on our ability to recruit patients to participate as well as the completion of required follow-up periods. Patients may be unwilling to participate in our gene therapy clinical trials because of negative publicity from adverse events related to the biotechnology or gene therapy fields, competitive clinical trials for similar patient populations, clinical trials in product candidates employing our vectors, the existence of current treatments or for other reasons. In addition, the indications that we are currently targeting and may in the future target are rare diseases, which may limit the pool of patients that may be enrolled in our ongoing or planned clinical trials. The timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed, which could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. For example, in 2017, the ongoing investigator-sponsored Phase 1 clinical trial of AVR-RD-01 encountered delays in the enrollment of patients due to delays in identifying patients for enrollment and the evaluation of information from screened potential trial participants. Patient enrollment and trial completion is affected by factors including the:

- size of the patient population and process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of gene therapy-based approaches to treatment of diseases, including any required pretreatment conditioning regimens;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor subjects adequately during and after treatment.

Our current product candidates are being developed to treat rare conditions. We plan to seek initial marketing approvals in the United States, Europe and certain other major markets, including Japan. We may not be able to initiate or continue clinical trials, including our recently initiated Phase 2 clinical trial for AVR-RD-01 for which enrollment is ongoing, if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by FDA or other foreign regulatory authorities. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for the conduct of clinical trials;
- the absence in some countries of established groups with sufficient regulatory expertise for review of gene therapy protocols;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical study sites;
- delays in obtaining required IRB approval at each clinical study site;
- delays in recruiting suitable patients to participate in our clinical studies;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical study operations or study sites;
- failure by our CROs, other third parties or us to adhere to clinical study requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical study sites or patients dropping out of a study;
- the occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues. In addition, if we make changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions, which could delay our clinical development plan or marketing approval for our product candidates. For example, while we are currently utilizing the LV1 version of the lentiviral vector in the ongoing Phase 1 and Phase 2 clinical trials of AVR-RD-01, we plan to transition our AVR-RD-01 lentiviral vectors to an LV2 version. While LV2 is intended to confer improvements in safety and efficiency in viral production, there is no guarantee that we can realize these intended benefits. In addition, the transition from LV1 to LV2 will likely require updates to our clinical trial applications and INDs with the relevant regulatory authorities, which may result in delay, suspension or termination of ongoing or future clinical trials pending our submission, and the agencies' review, of such updates. Clinical study delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If the results of our clinical studies are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling or a REMS that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical studies to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

Even if we complete the necessary preclinical and clinical studies, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate and the approval may be for a more narrow indication than we seek.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. If we are unable to obtain necessary regulatory approvals, our business, prospects, financial condition and results of operations may suffer.

While we intend to seek designations for our product candidates with the FDA and comparable foreign regulatory authorities that are intended to confer benefits such as a faster development process or an accelerated regulatory pathway, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

The FDA and comparable foreign regulatory authorities offer certain designations for product candidates that are designed to encourage the research and development of product candidates that are intended to address conditions with significant unmet medical need. These designations may confer benefits such as additional interaction with regulatory authorities, a potentially accelerated regulatory pathway and priority review. However, there can be no assurance that we will successfully obtain such designations for any of our product candidates. In addition, while such designations could expedite the development or approval process, they generally do not change the standards for approval. Even if we obtain such designations for one or more of our product candidates, there can be no assurance that we will realize their intended benefits.

For example, we may seek a Breakthrough Therapy Designation for some of our product candidates. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA are also eligible for accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification.

In addition, we may seek Fast Track Designation for some of our product candidates. If a therapy is intended for the treatment of a serious or life-threatening condition and the therapy demonstrates the potential to address unmet medical needs for this condition, the therapy sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, there can be no assurance that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures, and receiving a Fast Track Designation does not provide assurance of ultimate FDA approval. In addition, the FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program.

In addition, we may seek a regenerative medicine advanced therapy, or RMAT, designation for some of our product candidates. An RMAT is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells that lead to a durable modification of cells or tissues may meet the definition of a regenerative medicine therapy. The RMAT program is intended to facilitate efficient development and expedite review of RMATs, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A new drug application or a BLA for an RMAT may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support

accelerated approval. A regenerative medicine therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval. RMAT designation is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a regenerative medicine advanced therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of RMAT designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as for RMAT designation, the FDA may later decide that the biological products no longer meet the conditions for qualification.

Outside of the United States, we intend to develop AVR-RD-01 in Japan under the purview of the Japanese Pharmaceutical and Medical Device Agency, or PMDA. Pursuant to Japan's regenerative medicine law, an expedited path to conditional approval may exist for regenerative medicine products that show sufficient safety evidence and signals of efficacy in a Phase 2 clinical trial. However, there can be no assurance that the results of our recently initiated Phase 2 clinical trial will demonstrate the safety evidence and efficacy signals required for such conditional approval. In addition, this conditional approval is time-limited, and there must be an agreement as to follow-up collection of information to confirm efficacy and safety, similar to a post-marketing commitment in the United States.

We may be unable to obtain orphan drug designation for our product candidates and, even if we obtain such designation, we may not be able to realize the benefits of such designation, including potential marketing exclusivity of our product candidates, if approved.

Regulatory authorities in some jurisdictions, including the United States and other major markets, may designate drugs intended to treat conditions or diseases affecting relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

If we request orphan drug designation (or the foreign equivalent) for AVR-RD-01 or any of our other product candidates, there can be no assurances that the FDA or foreign regulatory authorities will grant any of our product candidates such designation. Additionally, the designation of any of our product candidates as an orphan product does not mean that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or foreign regulatory authorities from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug

designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition in the United States. Even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practices, or cGMP, requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;

- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and adversely affect our business, financial condition, results of operations and prospects.

In addition, the FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects.

We face significant competition in our industry and there can be no assurance that our product candidates, if approved, will achieve acceptance in the market over existing established therapies. In addition, our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our ability to successfully market or commercialize any of our product candidates.

We operate in a highly competitive segment of the biopharmaceutical market. We face competition from many different sources, including larger pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Our product candidates, if successfully developed and approved, will compete with established therapies, some of which are being marketed by large and international companies. In addition, we expect to compete with new treatments that are under development or may be advanced into the clinic by our competitors. There are a variety of product candidates, including gene therapies, in development for the indications that we are targeting.

We anticipate competing with the largest pharmaceutical companies in the world. For example, Sanofi and Shire market the enzyme replacement therapies, or ERTs, that represent the standard of care for Fabry patients. Recently, Amicus secured regulatory approval in Europe for its oral therapy for Fabry disease. For Gaucher disease, we expect to compete with existing enzyme replacement therapies marketed by Sanofi, Shire, Protalix and Pfizer, as well as oral therapies marketed by Actelion and Sanofi. Sanofi also markets an enzyme replacement therapy for Pompe disease. Cystinosis is currently treated by therapies marketed by Horizon Orphan, Mylan and Sigma Tau Pharmaceuticals. In addition, we may compete with other gene therapy companies in our industry such as bluebird bio and Spark Therapeutics.

Many of our competitors have significantly greater financial, product candidate development, manufacturing and marketing resources than we do. Large pharmaceutical and biotechnology companies have extensive experience in clinical testing and obtaining regulatory approval for their products, and mergers and acquisitions within these industries may result in even more resources being concentrated among a smaller

number of larger competitors. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel therapeutics or to in-license novel therapeutics that could make the product candidates that we develop obsolete. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our business would be materially and adversely affected if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, have broader market acceptance, are more convenient or are less expensive than any product candidate that we may develop.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances.

Our focus on developing our current product candidates may not yield any commercially viable products, and our failure to successfully identify and develop additional product candidates could impair our ability to grow.

As part of our growth strategy, we intend to identify, develop and market additional product candidates beyond our existing product candidates for Fabry disease, Gaucher disease, Pompe disease and cystinosis. We may spend several years completing our development of any particular current or future product candidates, and failure can occur at any stage. The product candidates to which we allocate our resources may not end up being successful. Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential than AVR-RD-01 or our other product candidates. Our spending on current and future research and development programs may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaborations, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate.

Because our internal research capabilities are limited, we may be dependent upon biotechnology companies, academic scientists and other researchers to sell or license product candidates, approved products or the underlying technology to us. The success of this strategy depends partly upon our ability to identify, select, discover and acquire promising product candidates and products.

In addition, certain of our current or future product candidates may not demonstrate in patients any or all of the pharmacological benefits we believe they may possess or compare favorably to existing, approved therapies, such as ERT. We have not yet succeeded and may never succeed in demonstrating efficacy and safety of our product candidates or any future product candidates in clinical trials or in obtaining marketing approval thereafter. For example, although we have evaluated AVR-RD-02, AVR-RD-03 and AVR-RD-04 in preclinical studies and have evaluated AVR-RD-01 in an early-stage clinical trial, we have not yet advanced AVR-RD-02, AVR-RD-03 and AVR-RD-04 into clinical trials or AVR-RD-01 into Phase 2 clinical development, nor have we obtained regulatory approval to sell any product based on our therapeutic approaches. Accordingly, our focus on treating these diseases may not result in the discovery and development of commercially viable products.

If we are unsuccessful in our development efforts, we may not be able to advance the development of our product candidates, commercialize products, raise capital, expand our business or continue our operations.

Risks related to our reliance on third parties

We expect to rely on third parties to conduct some or all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items. For example, we are moving our cell processing to an automated, closed system with a single third party supplier.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our preclinical and clinical studies are conducted in accordance with the study plan, protocols and regulatory requirements.

If our contract counterparties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies required to support approval of our product candidates or the FDA or other regulatory agencies may refuse to accept our clinical or preclinical data. For example, in 2017, the ongoing investigator-sponsored Phase 1 clinical trial of AVR-RD-01 encountered delays in the enrollment of patients due to delays in identifying patients for enrollment and the evaluation of information from screened potential trial participants.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to delays of our preclinical and clinical studies or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

We currently have relationships with a limited number of suppliers for the manufacturing of our viral vectors and product candidates. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in clinical studies must be manufactured in accordance with good manufacturing practices, or GMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's good laboratory practices, or GLP, and GMP regulations enforced by the FDA through its facilities inspection program. Some of our contract manufacturers have not produced a commercially-approved product and have never been inspected by the FDA before. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be materially harmed.

These factors could cause the delay of clinical studies, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our preclinical and clinical studies may be delayed.

We are dependent on a limited number of suppliers for some of our components and materials used in our product candidates.

We currently depend on a limited number of suppliers for some of the components necessary for our product candidates. We cannot be sure that these suppliers will remain in business, or that they will not be purchased by one of our competitors or another company that is not interested in continuing to produce these materials for our intended purpose. Our use of a limited number of suppliers of raw materials, components and finished goods exposes us to several risks, including disruptions in supply, price increases, late deliveries and an inability to meet customer demand. There are, in general, relatively few alternative sources of supply for these components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sale. Establishing additional or replacement suppliers for these components could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any supplier or manufacturing location could lead to supply delays or interruptions which would damage our business, financial condition, results of operations and prospects.

If we are required to switch to a replacement supplier, the manufacture and delivery of our product candidates could be interrupted for an extended period, adversely affecting our business. Establishing additional or replacement suppliers may not be accomplished quickly. If we are able to find a replacement supplier, the replacement supplier would need to be qualified and may require additional regulatory authority approval, which could result in further delay. For example, the FDA could require additional supplemental data and clinical trial data if we rely upon a new supplier. While we seek to maintain adequate inventory of the components and materials used in our product candidates, any interruption or delay in the supply of components or materials, or our inability to obtain components or materials from alternate sources at acceptable prices in a timely manner, could impair our ability to conduct our clinical trials and, if our product candidates are approved, to meet the demand of our customers and cause them to cancel orders.

In addition, as part of the FDA's approval of our product candidates, the FDA must review and approve the individual components of our production process, which includes the manufacturing processes and facilities of our suppliers. Our current suppliers have not undergone this process nor have they had any components included in any product approved by the FDA.

Our reliance on these suppliers subjects us to a number of risks that could harm our reputation, business, and financial condition, including, among other things:

- the interruption of supply resulting from modifications to or discontinuation of a supplier's operations;
- delays in product shipments resulting from uncorrected defects, reliability issues, or a supplier's variation in a component;
- a lack of long-term supply arrangements for key components with our suppliers;
- the inability to obtain adequate supply in a timely manner, or to obtain adequate supply on commercially reasonable terms;
- difficulty and cost associated with locating and qualifying alternative suppliers for our components in a timely manner;
- production delays related to the evaluation and testing of products from alternative suppliers, and corresponding regulatory qualifications;
- a delay in delivery due to our suppliers prioritizing other customer orders over ours;
- damage to our reputation caused by defective components produced by our suppliers;
- increased cost of our warranty program due to product repair or replacement based upon defects in components produced by our suppliers; and
- fluctuation in delivery by our suppliers due to changes in demand from us or their other customers.

If any of these risks materialize, costs could significantly increase and our ability to conduct our clinical trials and, if our product candidates are approved, to meet demand for our products could be impacted.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture our vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy approach, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets.

Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Risks related to commercialization of our product candidates

If we are unable to establish sales, distribution and marketing capabilities or enter into agreements with third parties to market and sell AVR-RD-01 and our other product candidates, we will be unable to generate any product revenue.

We currently have no sales, distribution or marketing organization. To successfully commercialize any of our current or future product candidates, if approved, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any product candidate we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding any approved product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates, if approved. Without an internal team or the support of a third-party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If the market opportunities for our product candidates are smaller than we believe they are, our product revenues may be adversely affected and our business may suffer.

We focus our research and product development on treatments for serious lysosomal storage diseases. Our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Even if we obtain any regulatory approval for our product candidates, the commercial success of our product candidates will depend in part on the medical community, patients, and third-party payors accepting gene therapy products in general, and our product candidates in particular, as effective, safe and cost-effective. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and others in the medical community. The degree of market acceptance of these product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the potential efficacy and potential advantages over alternative treatments;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the prevalence and severity of any side effects resulting from the conditioning regimen for the administration of our product candidates;
- the relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement.

Even if a product candidate displays a favorable efficacy and safety profile in preclinical and clinical studies, market acceptance of the product, if approved for commercial sale, will not be known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

We currently plan to conduct clinical trials for our product candidates outside of the United States, including in Canada, Australia, Japan, Europe and Israel. If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for any of our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford expensive treatments, such as stem cell transplants. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If reimbursement is not available, or is available only at limited levels, we may not be able to successfully commercialize our product candidates, if approved. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as the CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow the CMS to a substantial degree. It is difficult to predict what the CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and certain other major markets where we plan to commercialize may put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, efforts by governmental and third-party payors, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Due to the novel nature of our technology and the potential for our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates.

Our target patient populations are relatively small, as a result of which the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our product candidates, if approved.

Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs or otherwise adversely affect our business.

The manufacturing process we use to produce our product candidates is complex, novel and has not been validated for commercial use. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of our suppliers.

Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we and our manufacturing suppliers employ multiple steps to control the manufacturing process with the goal of ensuring that the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, including even minor deviations from the intended process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA or other applicable regulatory standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Even slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. There is no assurance we will not experience lot failures in the future. Lot failures or product recalls could cause us to delay clinical trials, or, if approved, commercial product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

Healthcare legislative reform measures and constraints on national budget social security systems may have a material adverse effect on our business and results of operations.

Third-party payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act or ACA or PPACA, as amended by the Health Care and Education Reconciliation Act of 2010, or the ACA, was enacted, which, among other things, subjected biologic products to potential competition by lower-cost biosimilars, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug

Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research. Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial and Congressional challenges. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, the CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Congress may consider other legislation to replace elements of the ACA.

The Tax Cuts and Jobs Act of 2017, or TCJA, includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device exercise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to reduce the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Congress also could consider subsequent legislation to replace elements of the ACA that are repealed. Thus, the full impact of the ACA, any law replacing elements of it, and the political uncertainty surrounding any repeal or replacement legislation on our business remains unclear. In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012, was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our drug product candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;

- the level of taxes that we are required to pay; and
- the availability of capital.

Any denial in coverage or reduction in reimbursement from Medicare or other government programs may result in a similar denial or reduction in payments from private payors, which may adversely affect our future profitability.

Any contamination in our manufacturing process, shortages of materials or failure of any of our key suppliers to deliver necessary components could result in interruption in the supply of our product candidates and delays in our clinical development or commercialization schedules.

Given the nature of biologics manufacturing, there is a risk of contamination in our manufacturing processes. Any contamination could materially adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the materials required in our manufacturing process are derived from biologic sources. Such materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Risks related to our business operations

Our gene therapy approach utilizes lentiviral vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology, with only a limited number of gene therapy products approved to date. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Adverse events in our clinical studies, even if not ultimately attributable to our product candidates (such as the many adverse events that typically arise from the conditioning process), or adverse events in other lentiviral gene therapy trials, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on principal members of our executive team and key employees, including our Chief Executive Officer, Chief Financial Officer, Head of Operations, Chief Science Officer, Chief Business Officer, and Chief Medical Officer, the loss of whose services may adversely impact the achievement of our

objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. We do not maintain "key person" insurance policies on the lives of these individuals or the lives of any of our other employees. The loss of the services of one or more of our current employees might impede the achievement of our research, development and commercialization objectives. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives in our industry, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials may make it more challenging to recruit and retain qualified personnel. The inability to recruit or the loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

We will need to expand our operations and we may experience difficulties in managing this growth, which could disrupt our operations.

As of June 1, 2018, we had 34 full-time employees. As we mature, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA or of other foreign regulatory authorities, provide accurate information to the FDA and other foreign regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the

United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

Among other matters, U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, which are collectively referred to as Trade Laws, prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase in time. We plan to engage third parties for clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals and we can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities. The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the United States Foreign Corrupt Practices Act's accounting provisions.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws health information privacy and security laws, and other health care laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Act and Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, the purchase, lease, order, or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the

Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act or federal civil money penalties;

- federal civil and criminal false claims laws and civil monetary penalty laws, such as the federal False Claims Act, which impose criminal and civil penalties and authorize civil whistleblower or qui tam actions, against individuals or entities for, among other things: knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent; making, using or causing to be made or used, a false statement of record material to a false or fraudulent claim or obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government;
- the anti-inducement law, which prohibits, among other things, the offering or giving of remuneration, which includes, without limitation, any transfer of items or services for free or for less than fair market value (with limited exceptions), to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary's selection of a particular supplier of items or services reimbursable by a federal or state governmental program;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information relating to the privacy, security and transmission of individually identifiable health information;
- the federal transparency requirements under the Affordable Care Act, including the provision commonly referred to as the Physician Payments Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members;
- federal government price reporting laws, which require us to calculate and report complex pricing metrics in an accurate and timely manner to government programs; and
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers

Additionally, we are subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payer. Many U.S. states

have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payers, including private insurers. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties. Finally, there are state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use of our product candidates in clinical studies and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- the impairment of our business reputation;
- the withdrawal of clinical study participants;
- costs due to related litigation;
- the distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of $5.0 million per occurrence and $5.0 million in the aggregate. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by certain of our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life- threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially adversely affect our business, financial condition, results of operations and prospects.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payors or our collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business, financial condition, results of operations and prospects.

We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite our security measures, our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. For example, in 2017 we were subjected to a cyberattack by a third party, which led to the theft of a portion of our funds. We implemented remedial measures promptly following this breach and do not believe that this breach had a material adverse effect on our business. However, if any cyberattack or data breach were to occur in the future and cause interruptions in our or our collaborators', contractors' or consultants' operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

Comprehensive tax reform legislation could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law the TCJA that significantly reforms the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contains significant changes to corporate taxation, including the reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), the limitation of the deduction for net operating losses to 80% of current year taxable income and the elimination of net operating loss carrybacks and modification or repeal of many business deductions and credits (including the reduction of the business tax credit for certain clinical testing expenses incurred in the testing of certain drugs for rare diseases or conditions generally referred to as "orphan drugs"). We continue to examine the impact this tax reform legislation may have on our business. However, the effect of the TCJA on our business, whether adverse or favorable, is uncertain, and may not become evident for some period of time. We urge investors to consult with their legal and tax advisers regarding the implications of the TCJA on an investment in our common stock.

We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.

As of December 31, 2017, we had federal and state net operating loss carryforwards of $19.0 million and $18.9 million, respectively, and state research and development tax credit carryforwards of approximately

$119,000. If not utilized, the net operating loss carryforwards and research and development credits will generally expire at various dates through 2037. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. In addition, under Section 382 of the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We may have experienced ownership changes in the past. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership, including this offering, some of which may be outside of our control. If an ownership change occurred or occurs and our ability to use our historical net operating loss and tax credit carryforwards is materially limited, or if our research and development carryforwards are adjusted, it would harm our future operating results by effectively increasing our future tax obligations. The reduction of the corporate tax rate under the TCJA may cause a reduction in the economic benefit of our net operating loss carryforwards and other deferred tax assets available to us. Under the TCJA, net operating losses generated after December 31, 2017 will not be subject to expiration.

Risks related to our intellectual property

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we or our licensors are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. In particular, we are aware of issued patents in the United States that cover the lentiviral vectors used in the manufacture of our product candidates. While we believe that we have reasonable defenses against a claim of infringement, potentially including that certain of these patents are expected to expire prior to commercializing our product candidates, if approved, in the United States, there can be no assurance that we will prevail in any such action by the holder of these patents. In the event that the holder of these patents seeks to enforce its patent rights and our defenses against a claim of infringement are unsuccessful, we may not be able to commercialize our product candidates in the United States, if approved, without first obtaining a license to some or all of these patents, which may not be available on commercially reasonable terms or at all. In addition, the defense of any claim of infringement, even if successful, is time-consuming, expensive and diverts the attention of our management from our ongoing business operations.

Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe or be alleged to infringe. In addition, third parties may obtain patents in the future and claim that use of our or our licensors' technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Even in the absence of a finding of infringement, we may choose to obtain a license, if such a license is available. A successful claim of patent or other intellectual property infringement against us could materially adversely affect our business, results of operations and financial condition.

Our rights to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We depend upon the intellectual property rights granted to us under licenses from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our gene therapy product candidates. In particular, we have in-licensed certain intellectual property rights and know-how from the University Health Network (relevant to AVR-RD-01 and our Fabry program) and affiliates of Lund University (relevant to AVR-RD-02 and our Gaucher program). In addition, we have in-licensed patents and patent applications from BioMarin Pharmaceutical Inc., or Biomarin (relevant to AVR-RD-03 and our Pompe program), and GenStem Therapeutics Inc., or GenStem (relevant to AVR-RD-04 and our cystinosis program), directed to compositions and methods related to the manufacture and use of AVR-RD-03 and AVR-RD-04, respectively. Any termination of these licenses could result in the loss of significant rights and could harm or prevent our ability to commercialize our product candidates.

Each of our existing licenses are exclusive but are limited to particular fields, such as Fabry disease, Gaucher disease, Pompe disease, or cystinosis, and are subject to certain retained rights. Absent an amendment or additional agreement, we may not have the right to use intellectual property in-licensed for one of our programs for another program. In addition, licenses that we may enter into in the future may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. Licenses to additional third-party technology that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could have a material adverse effect on our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. For example, pursuant to each of our intellectual property licenses with GenStem, BioMarin, and the rights holders associated with Lund University, our licensors retain control of such activities. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected.

Our current license agreements impose, and we expect that future license agreements that we may enter into will impose, various obligations, including diligence and certain payment obligations. If we fail to satisfy our

obligations, the licensor may have the right to terminate the agreement. Disputes may arise between us and any of our licensors regarding intellectual property subject to such agreements and other issues. Such disputes over intellectual property that we have licensed or the terms of our license agreements may prevent or impair our ability to maintain our current arrangements on acceptable terms, or at all, or may impair the value of the arrangement to us. Any such dispute could have a material adverse effect on our business. If we cannot maintain a necessary license agreement or if the agreement is terminated, we may be unable to successfully develop and commercialize the affected product candidates.

If we are unable to obtain and maintain patent protection for our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected.

Our ability to compete effectively will depend, in part, on our ability to maintain the proprietary nature of our technology and manufacturing processes. We rely on manufacturing and other know-how, patents, trade secrets, trademarks, license agreements and contractual provisions to establish our intellectual property rights and protect our products. These legal means, however, afford only limited protection and may not adequately protect our rights. The failure to obtain, maintain, enforce or defend such intellectual property rights, for any reason, could allow third parties to make competing products or impact our ability to develop, manufacture and market our products, if approved, on a commercially viable basis, or at all, which could have a material adverse effect on our financial condition and results of operations.

In particular, we rely primarily on trade secrets, know-how and other unpatented technology, which are difficult to protect. Although we seek such protection in part by entering into confidentiality agreements with our vendors, employees, consultants and others who may have access to proprietary information, we cannot be certain that these agreements will not be breached, adequate remedies for any breach would be available or our trade secrets, know-how and other unpatented proprietary technology will not otherwise become known to or be independently developed by our competitors. If we are unsuccessful in protecting our intellectual property rights, sales of our products may suffer and our ability to generate revenue could be severely impacted.

Our licensors and we have sought, and we intend to continue to seek to protect our proprietary position by filing patent applications in the United States and, in at least some cases, one or more countries outside the United States related to current and future product candidates that are important to our business. However, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents, whether the claims of any issued patents will provide us with a competitive advantage, or whether we will be able to successfully pursue patent applications in the future related to our current or future product candidates. We currently have no owned or inlicensed patents or patent applications covering AVR-RD-01 or AVR-RD-02, and the patent application that we in-licensed related to AVR-RD-04 is at a very early stage. Many of our product candidates are in-licensed from third parties. Accordingly, in some cases, the availability and scope of potential patent protection is limited based on prior decisions by our licensors or the inventors, such as decisions on when to file patent applications or whether to file patent applications at all.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although our license agreements grant us worldwide rights, and our currently in-licensed U.S. patent rights have certain corresponding foreign patents or patent applications, there can be no assurance that we will obtain or maintain such corresponding patents or patent applications with respect to any future license agreements. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States even in jurisdictions where we and our licensors pursue patent protection. Consequently, we and our licensors may not be able to

prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we and our licensors pursue patent protection, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights, even if obtained, in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensing partners or we initiate legal proceedings against a third-party to enforce a patent covering one of our product candidates, should such a patent issue, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the United States Patent and Trademark Office, or USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or

have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management. Our licensors may face similar risks, which could have an adverse impact on intellectual property that is licensed to us.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

We may be subject to claims challenging the inventorship or ownership of the patents and other intellectual property that we own or license.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an ownership interest in the patents and intellectual property that we own or license or that we may own or license in the future. While it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own; our licensors may face similar obstacles. We could be subject to ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against any claims challenging inventorship or ownership. If we or our licensors fail in defending any such claims, we may have to pay monetary damages and may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property, which could adversely impact our business, results of operations and financial condition.

Some intellectual property which we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "marchin" rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with nonU.S. manufacturers.

Some of the intellectual property rights we have licensed, including rights licensed to us by GenStem, may have been generated through the use of U.S. government and California state funding and may therefore be

subject to certain federal and state laws and regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply. Any exercise by the government of certain of its rights could harm our competitive position, business, financial condition, results of operations and prospects. With respect to state funding, specifically funding via the California Institute of Regenerative Medicine, or CIRM, the grantee has certain obligations and the state or CIRM has certain rights. For example, the grantee has an obligation to share intellectual property, including research results, generated by CIRM-funded research, for research use in California.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

Changes in either the patent laws or the interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a "first-to-invent" system to a "first-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court of the United States, or Supreme Court. On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc., or

Prometheus, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as “administering” or “determining” steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to not patent-eligible subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc., or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent-eligible. On March 4, 2014, the USPTO issued a guidance memorandum to patent examiners entitled 2014 Procedure For Subject Matter Eligibility Analysis Of Claims Reciting Or Involving Laws Of Nature/Natural Principles, Natural Phenomena, And/Or Natural Products. These guidelines instruct USPTO examiners on the ramifications of the Prometheus and Myriad rulings and apply the Myriad ruling to natural products and principles including all naturally occurring nucleic acids.

Certain claims of our licensed patents and patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties. In addition, the 2014 USPTO guidance could impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our product candidates will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court’s decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have a material adverse effect on our existing patent rights and our ability to protect and enforce our intellectual property in the future.

Moreover, although the Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more U.S. patents that we license or may own or license in the future, if any, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method

for manufacturing it may be extended. A patent may only be extended once and only based on a single approved product. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially. In addition, we do not control the efforts of our licensors to obtain a patent term extension, and there can be no assurance that they will pursue or obtain such extensions to the patents that we license from them.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We currently do not have trademarks or trademark applications with the USPTO for the mark "AVRO" and the AVROBIO logo. In the future, even if we apply for registration of these marks, there can be no assurance that such registration will be approved. Once registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we license or may own or license in the future;
- we, our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patents or pending patent applications that we license or may own or license in the future;
- we, our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending licensed patent applications or those that we may own or license in the future will not lead to issued patents;
- issued patents that we hold rights to or may hold rights to in the future may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- one or more of our product candidates may never be protected by patents;

- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and
- we may choose not to file a patent application for certain trade secrets or know-how, and a third party may subsequently file a patent application or obtain a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Risks related to this offering and ownership of our common stock

The market price of our common stock may be highly volatile, and you may not be able to resell your shares at or above the initial public offering price.

Our stock price is likely to be volatile. The stock market in general, and the market for biopharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- adverse results or delays in preclinical studies or clinical trials;
- reports of adverse events in other gene therapy products or clinical studies of such products;
- an inability to obtain additional funding;
- failure by us to successfully develop and commercialize our product candidates;
- failure by us to maintain our existing strategic collaborations or enter into new collaborations;
- failure by us or our licensors and strategic partners to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;
- an inability to obtain adequate product supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- the introduction of new products, services or technologies by our competitors;
- failure by us to meet or exceed financial projections we may provide to the public;
- failure by us to meet or exceed the financial projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;

- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and
- the trading volume of our common stock.

In addition, companies trading in the stock market in general, and The Nasdaq Global Select Market in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

An active trading market for our common stock may not develop.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters. Although we have been approved to list our common stock on The Nasdaq Global Select Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares, or at all.

An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling additional shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common stock will likely depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. We do not currently have research coverage, and there can be no assurance that analysts will cover us, or provide favorable coverage. Securities or industry analysts may elect not to provide research coverage of our common stock after this offering, and such lack of research coverage may negatively impact the market price of our common stock. In the event we do have analyst coverage, if one or more analysts downgrade our stock or change their opinion of our stock, our share price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers, directors, five percent stockholders and their affiliates beneficially own approximately 79.20% of our voting stock and, upon closing of this offering, that same group will beneficially own approximately 61.30% of our outstanding voting stock not accounting for any shares purchased in this offering by certain of our existing stockholders, including certain affiliates of our directors, who have agreed to purchase an aggregate of approximately $23 million of our common stock in this offering. As a result, if these stockholders were to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affair. For example, these stockholders, acting together, may be able to

control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders. Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the price at which shares are being sold in this offering and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors or they may want us to pursue strategies that deviate from the interests of other stockholders.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," or EGC, as defined in the JOBS Act. We will remain an EGC until the earliest of: (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC, which means the first day of the year following the first year in which the market value of our common stock that is held by non-affiliates exceeds $700 million as of June 30. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, or Section 404;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" disclosure;
- reduced disclosure obligations regarding executive compensation; and
- an exemption from the requirement to seek nonbinding advisory votes on executive compensation or golden parachute arrangements.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in this prospectus. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an EGC may take advantage of an extended transition period for complying with new or revised accounting standards. This allows an EGC to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, and particularly after we are no longer an EGC, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of

2002 and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our stock.

We will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an EGC, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We could be an EGC for up to five years. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management’s assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation.

We and our independent registered public accounting firm have identified material weaknesses in our internal control over financial reporting. If we are unable to remedy these material weaknesses, or if we fail to establish and maintain effective internal controls, we may be unable to produce timely and accurate financial statements, and we may conclude that our internal control over financial reporting is not effective, which could adversely impact our investors' confidence and our stock price.

In connection with the audit of our consolidated financial statements for the years ended December 31, 2016 and 2017, we and our independent registered public accounting firm identified material weaknesses in our internal control over financial reporting related to deficiencies in our controls over the financial statement close and cash disbursement processes. Specifically, there was a lack of controls over the identification and review of complex accounting issues involving significant judgment or estimates as well as the cutoff and classification of certain expenses between general and administrative and research and development. In addition, our internal controls related to the cash disbursements process were not adequately designed to identify unauthorized payment requests. Specifically, in 2017 we were subject to a cyberattack by a third party. This deficiency in our controls resulted in the theft of a portion of our funds.

We are implementing measures designed to improve our internal control over financial reporting to remediate these material weaknesses, including formalizing our processes and internal control documentation and strengthening supervisory reviews by our financial management; hiring additional qualified accounting and finance personnel and engaging financial consultants to enable the implementation of internal control over financial reporting and segregating duties amongst accounting and finance personnel; and planning to implement certain accounting systems to automate manual processes, such as tracking and accounting for stock-based awards.

We expect to incur additional costs to remediate these control deficiencies, though there can be no assurance that our efforts will be successful or avoid potential future material weaknesses. If we are unable to successfully remediate our existing or any future material weaknesses in our internal control over financial reporting, or if we identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the Securities and Exchange Commission, or SEC, or other regulatory authorities.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon completion of this offering, we will become subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the pro forma book value per share of our tangible assets after subtracting our liabilities. As a result,

investors purchasing shares of common stock in this offering will incur immediate dilution of $12.80 per share, based on the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, and our pro forma net tangible book value as of March 31, 2018. Further, investors purchasing shares of common stock in this offering will contribute approximately 53.1% of the total amount invested by stockholders since our inception, but will own only approximately 22.7% of the shares of common stock outstanding. For information on how the foregoing amounts were calculated, see "Dilution."

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly.

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to certain restrictions described below. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding 23,149,645 shares of common stock based on the number of shares outstanding as of March 31, 2018 assuming the conversion of our preferred stock upon the closing of this offering, (or 23,936,838 shares if the underwriters exercise their option to purchase additional shares in full). This includes the 5,247,958 shares that we are selling in this offering (or 6,035,151 shares if the underwriters exercise their option to purchase additional shares in full), which may be resold in the public market immediately without restriction, unless purchased by our affiliates. The remaining 17,901,687 shares currently are restricted as a result of securities laws or lock-up agreements but will be able to be sold after the offering as described in the "Shares eligible for future sale" and "Underwriters" sections of this prospectus. Moreover, after this offering, holders of an aggregate of approximately 15.3 million shares of our common stock will have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. In addition, 1,788,750 shares reserved for issuance upon the exercise of existing stock options outstanding as of March 31, 2018 under our current equity incentive plan will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. We intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriters" section of this prospectus.

In addition, Morgan Stanley & Co. LLC and Cowen and Company, LLC may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. Sales of a substantial number of such shares upon expiration of the lock-up agreements, the perception that such sales may occur, or early release of these agreements, could cause our market price to fall or make it more difficult for you to sell your common stock at a time and price that you deem appropriate.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled "Use of proceeds," and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock. For example, our loan and security agreement with Silicon Valley Bank restricts our ability to pay any dividends or making any distributions on account of our capital stock. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Provisions in our amended and restated certificate of incorporation and by-laws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Our amended and restated certificate of incorporation, amended and restated by-laws and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and by-laws, which will become effective upon the closing of this offering, include provisions that:

- authorize “blank check” preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors, the chairperson of our board of directors, our chief executive officer or our president;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that our directors may be removed only for cause;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated by-laws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated by-laws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us.

Any provision of our amended and restated certificate of incorporation or amended and restated by-laws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our amended and restated bylaws limit the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of or based on a fiduciary duty owed by any of our current or former directors, officers or other employee to us or our stockholders, (iii) any action asserting a claim against us or any of our current or former directors, officers, employees or stockholders arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions which could have an adverse effect on our business, financial condition or results of operations. In addition, our amended and restated bylaws contain a provision by virtue of which unless we consent in writing to the selection of an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for any complaint asserting a cause of action arising under the Securities Act. We have chosen the United States Court for the District of Massachusetts as the exclusive forum for such causes of action because our principal executive offices are located in Cambridge, Massachusetts. The U.S. District Court in Massachusetts may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders. In addition, some companies that have adopted similar federal district court forum selection provisions are currently subject to a suit in the Court of Chancery of the State of Delaware brought by stockholders who assert that the federal district court forum selection provision is not enforceable. We recognize that the federal district court forum selection clause may impose additional litigation costs on stockholders who assert the provision is not enforceable and may impose more general additional litigation costs in pursuing any such claims, particularly if the stockholders do not reside in or near the Commonwealth of Massachusetts. See "Description of Capital Stock—Choice of Forum."

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the sections entitled “Prospectus Summary,” “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Business,” contains express or implied forward-looking statements that are based on our management’s belief and assumptions and on information currently available to our management. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the timing, progress and results of preclinical studies and clinical trials for our programs and product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing, scope or likelihood of regulatory filings and approvals;
- our ability to develop and advance product candidates into, and successfully complete, clinical studies;
- our expectations regarding the size of the patient populations for our product candidates, if approved for commercial use;
- the implementation of our business model and our strategic plans for our business, product candidates and technology, including our transition to LV2 and our use of a milder conditioning regimen;
- our commercialization, marketing and manufacturing capabilities and strategy;
- the pricing and reimbursement of our product candidates, if approved;
- the scalability and commercial viability of our manufacturing methods and processes, including our plans to move to a closed, automated system;
- the rate and degree of market acceptance and clinical utility of our product candidates, in particular, and gene therapy, in general;
- our ability to establish or maintain collaborations or strategic relationships or obtain additional funding;
- our competitive position;
- the scope of protection we and/or our licensors are able to establish and maintain for intellectual property rights covering our product candidates;
- developments and projections relating to our competitors and our industry;
- our expectations related to the use of proceeds from this offering;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our ability to remediate the material weaknesses that we and our independent registered public accounting firm identified and avoid any findings of material weaknesses or significant deficiencies in the future;
- the impact of laws and regulations;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act; and
- other risks and uncertainties, including those listed under the caption “Risk Factors”

In some cases, forward-looking statements can be identified by terminology such as “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue” or the negative of these terms or other comparable terminology. These statements are only predictions. You should not place undue reliance on forward-looking statements because they involve known and unknown risks, uncertainties and other factors, which are, in some cases, beyond our control and which could materially affect results. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section entitled “Risk Factors” and elsewhere in this prospectus. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this prospectus and the documents that we reference in this prospectus and have filed with the Securities and Exchange Commission as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should therefore not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of common stock in this offering will be approximately $90.5 million, or approximately $104.4 million if the underwriters exercise their over-allotment option in full, based upon the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents of $57.9 million as of March 31, 2018, as follows:

- approximately $13.4 million to fund expenses for our lead product candidate, AVR-RD-01, for the treatment of Fabry disease in our ongoing Phase 2 clinical trial and to support the ongoing investigator-sponsored Phase 1 clinical trial;
- approximately $12.9 million to fund expenses to advance AVR-RD-02 for the treatment of Gaucher disease into Phase 1/2 clinical trials;
- approximately $3.2 million to fund expenses to advance AVR-RD-03 for Pompe disease further into preclinical development;
- approximately $5.4 million to fund expenses to advance AVR-RD-04 for the treatment of cystinosis, including to support the planned initial investigator-sponsored Phase 1/2 clinical trial;
- approximately $28.0 million to fund expenses for our external and internal manufacturing and process development activities related to the advancement of our product candidates;
- approximately $32.6 million to fund research and development activities that relate to all of our clinical and preclinical activities, including the cost of research and development personnel; and
- the remainder for planned general and administrative expenses, the costs of operating as a public company, working capital and other general corporate purposes.

Based on our current plans, we expect that the proceeds allocated as described above will be sufficient to complete the ongoing Phase 1 and Phase 2 clinical trials for AVR-RD-01, but will be insufficient to complete the above referenced trials and studies for AVR-RD-02, AVR-RD-03 and AVR-RD-04 or subsequent clinical trials of AVR-RD-01. Based on our current plans, we believe our existing cash and cash equivalents, together with the net proceeds from this offering, will be sufficient to fund our operations into 2020.

This expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. For example, we may use a portion of the net proceeds for the acquisition of businesses or technologies to continue to build our pipeline, our research and development capabilities and our intellectual property position, although we currently have no agreements, commitments or understandings with respect to any such transaction. We cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the timing and plans for initiation of our planned clinical trials, the progress of our research and development, the status of and results from non-clinical studies or clinical trials we may commence in the future, as well as any collaborations that we may enter into with third parties for our product candidates or strategic opportunities that become available to us, and any unforeseen cash needs.

Our management will have broad discretion in the application of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of the net proceeds from this offering. The timing and amount of our actual expenditures will be based on many factors, including cash flows from operations and the anticipated growth of our business. Pending these uses, we plan to hold these net proceeds in non-interest bearing accounts, with the goal of capital preservation and liquidity so that such funds are readily available to fund our operations.

DIVIDEND POLICY

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future. Any future determination related to dividend policy will be made at the discretion of our board of directors and will depend on, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant. Investors should not purchase our common stock with the expectation of receiving cash dividends. In addition, under our loan and security agreement with Silicon Valley Bank, we are restricted from paying any dividends or making any distributions on account of our capital stock. Moreover, the terms of any future indebtedness that we may incur could restrict our ability to pay dividends. See “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources” for a description of the restrictions on our ability to pay dividends.

CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of March 31, 2018:

- on an actual basis;
- on a pro forma basis to give effect to:
  - the conversion of all outstanding shares of preferred stock into an aggregate of 15,320,213 shares of common stock upon the closing of this offering;
  - the conversion of our warrant to purchase preferred stock into a warrant to purchase common stock upon the closing of this offering;
  - the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering; and
- on a pro forma as adjusted basis giving effect to the pro forma adjustments set forth above and to give further effect (i) to our issuance and sale of 5,247,958 shares of common stock in this offering at the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, and (ii) the payment by us of an aggregate of $2.0 million to University Health Network pursuant to the Stock Purchase Agreement, dated as of January 27, 2016, between us and University Health Network, in connection with this offering. Additionally, for purposes of the pro forma as adjusted amounts shown below, the net proceeds to be received by us from the sale of common stock in this offering have been increased by approximately $55,000 to reflect the estimated offering expenses that had been paid by us as of March 31, 2018.

You should read the following table in conjunction with our consolidated financial statements and the related notes appearing elsewhere in this prospectus, and the sections of this prospectus titled “Use of Proceeds,” “Selected Consolidated Financial Data,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Description of Share Capital.”

	As of March 31, 2018		
	Actual	Pro Forma	Pro Forma As Adjusted(1)
	(in thousands, except share and per share data)		
Cash and cash equivalents	$ 57,928	$ 57,928	$ 146,438
Warrant to purchase redeemable preferred stock	$ 47	$ —	$ —
Redeemable convertible preferred stock (Series Seed, A and B), $0.0001 par value; 63,491,857 shares authorized, 63,303,154 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	87,500	—	—
Stockholders’ (deficit) equity:			
Preferred stock, $0.0001 par value, no share authorized, issued or outstanding, actual; 10,000,000 shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.0001 par value; 82,000,000 shares authorized, 2,581,474 shares issued and 2,335,926 shares outstanding, actual; 150,000,000 shares authorized, 17,901,687 shares issued and 17,656,139 shares outstanding, pro forma; 150,000,000 shares authorized, 23,149,645 shares issued and 22,904,097 shares outstanding, pro forma as adjusted	—	2	2
Additional paid-in capital	109	87,654	178,109
Accumulated deficit	(33,620)	(33,620)	(34,662)
Total stockholders’ (deficit) equity	(33,511)	54,036	143,449
Total capitalization	$ 54,036	$ 54,036	$ 143,449

(1)

The number of shares of common stock outstanding in the table above does not include:

- 1,788,750 shares of common stock issuable upon the exercise of stock options outstanding as of March 31, 2018, with a weighted average exercise price of $2.56 per share;
- 6,850 shares of common stock issuable upon the exercise of a warrant to purchase preferred stock outstanding as of March 31, 2018 that will automatically become a warrant to purchase common stock upon the completion of this offering, with an exercise price of $3.2845 per share;
- an additional 58,472 shares of common stock reserved for issuance under our Amended and Restated 2015 Stock Option and Grant Plan as of March 31, 2018, which shares will no longer be reserved following this offering;
- an additional 616,300 shares of common stock that were made available for future issuance under our 2018 Stock Option and Grant Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- an additional 223,200 shares of common stock that were made available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per common share immediately after this offering.

Our historical net tangible book (deficit) as of March 31, 2018 was $33.5 million, or $(12.98) per share of common stock. Our historical net tangible book (deficit) is the amount of our total tangible assets less our total liabilities and the carrying value of redeemable convertible preferred stock, which is not included within stockholders' (deficit). Historical net tangible book (deficit) per share represents historical net tangible book (deficit) divided by the 2,581,474 shares of our common stock outstanding as of March 31, 2018.

Our pro forma net tangible book value as of March 31, 2018 was $54.0 million, or $3.02 per share of common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to:

- the conversion of all outstanding shares of redeemable convertible preferred stock into an aggregate of 15,320,213 shares of common stock upon the closing of this offering; and
- the conversion of our warrant to purchase preferred stock into a warrant to purchase common stock upon the closing of this offering.

Pro forma net tangible book value per share represents our pro forma net tangible book value divided by the total number of shares of our common stock outstanding as of March 31, 2018, after giving effect to the pro forma adjustments described above.

After giving further effect to (i) our issuance and sale of 5,247,958 shares of common stock in this offering at the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and offering expenses payable by us, and (ii) the payment by us of an aggregate of $2.0 million to University Health Network pursuant to the Stock Purchase Agreement, dated as of January 27, 2016, between us and University Health Network, in connection with this offering, our pro forma as adjusted net tangible book value as of March 31, 2018 would have been $143.4 million, or $6.20 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $3.18 to existing shareholders and immediate dilution in pro forma as adjusted net tangible book value per share of $12.80 to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Initial public offering price per share		$19.00
Historical net tangible book value (deficit) per share as of March 31, 2018	$(12.98)	
Increase per share attributable to the pro forma adjustments described above	16.00	
Pro forma net tangible book value per share as of March 31, 2018	3.02	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing common stock in this offering	3.18	
Pro forma as adjusted net tangible book value per share after this offering		6.20
Dilution in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering		$12.80

If the underwriters exercise their over-allotment option in this offering in full, the pro forma as adjusted net tangible book value per share after this offering would be $6.57 per share, and the dilution in pro forma as adjusted net tangible book value per share to new investors purchasing common shares in this offering would be $12.43 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, on the pro forma as adjusted basis described above as of March 31, 2018, the total number of shares of common stock purchased from us, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing shareholders and by new investors in this offering at the initial public offering price of $19.00 per share before deducting underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percentage	Amount	Percentage	
Existing shareholders	17,901,687	77.3%	$ 88,183,696	46.9%	$ 4.93
New investors	5,247,958	22.7	99,711,202	53.1	$ 19.00
Total	23,149,645	100.0%	$187,894,898	100.0%	

Certain of our existing stockholders, including certain affiliates of our directors, have agreed to purchase an aggregate of approximately $23 million of shares of our common stock in this offering at the initial public offering price. The table and discussion above do not give effect to the purchases by such investors.

The table above assumes no exercise of the underwriters' over-allotment option in this offering. If the underwriters' over-allotment option is exercised in full, the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 25.2% of the total number of shares of common stock outstanding after this offering, and the number of shares held by existing shareholders would be reduced to 74.8% of the total number of shares of common stock outstanding after this offering.

The tables and discussion above do not include:

- 1,788,750 shares of common stock issuable upon the exercise of stock options outstanding as of March 31, 2018, with a weighted average exercise price of $2.56 per share;
- 6,850 shares of common stock issuable upon the exercise of a warrant to purchase preferred stock outstanding as of March 31, 2018 that will automatically become a warrant to purchase common stock upon the completion of this offering, with an exercise price of $3.2845 per share;
- an additional 58,472 shares of common stock reserved for issuance under our Amended and Restated 2015 Stock Option and Grant Plan as of March 31, 2018, which shares will no longer be reserved following this offering;
- an additional 616,300 shares of common stock that were made available for future issuance under our 2018 Stock Option and Grant Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- an additional 223,200 shares of common stock that were made available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

To the extent that stock options or warrants are exercised, new stock options are issued under our equity incentive plans, or we issue additional shares of common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

SELECTED CONSOLIDATED FINANCIAL DATA

The selected consolidated statements of operations data for the years ended December 31, 2016 and 2017 and the selected consolidated balance sheet data as of December 31, 2016 and 2017 are derived from our audited financial statements included elsewhere in this prospectus. The consolidated statements of operations data for the three months ended March 31, 2017 and 2018 and the consolidated balance sheet data as of March 31, 2018 have been derived from our unaudited condensed consolidated financial statements included elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information contained in those statements. Our historical results are not necessarily indicative of the results that may be expected in any future period. You should read the following selected financial data together with the information in the sections titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus.

	Year Ended December 31,		Three Months Ended March 31,	
	2016	2017	2017	2018
	(in thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Operating expenses:				
Research and development	$ 2,663	$ 15,191	$ 1,434	$ 5,647
General and administrative	1,962	3,195	610	2,141
Total operating expenses	4,625	18,386	2,044	7,788
Loss from operations	(4,625)	(18,386)	(2,044)	(7,788)
Other income (expense):				
Interest income	6	57	4	158
Change in fair value of preferred stock warrant liability	—	(17)	—	(12)
Change in fair value of derivative liability	(39)	(283)	(32)	(587)
Other expense	(6)	(19)	(5)	(13)
Total other expense, net	(39)	(262)	(33)	(454)
Net loss	$ (4,664)	$ (18,648)	$ (2,077)	$ (8,242)
Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	$ (4,664)	$ (18,648)	$ (2,077)	$ (8,242)
Accretion of redeemable convertible preferred stock to redemption value	(305)	(85)	(47)	(2,243)
Net loss attributable to common stockholders	$ (4,969)	$ (18,733)	$ (2,124)	$ (10,485)
Net loss per share attributable to common stockholders—basic and diluted(1)	$ (2.44)	$ (8.38)	$ (0.97)	$ (4.51)
Weighted-average common shares outstanding—basic and diluted(1)	2,038,025	2,235,865	2,181,715	2,324,790
Pro forma net loss per share attributable to common stockholders—basic and diluted(1)		$ (2.69)		$ (0.51)
Pro forma weighted-average common shares outstanding—basic and diluted(1)		6,922,173		16,187,901

(1) See Notes 2 and 13 to our audited consolidated financial statements and Note 12 to our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus for further details on the calculation of basic and diluted net loss per share attributable to common stockholders and on the calculation of pro forma basic and diluted net loss per share attributable to common stockholders.

	As of December 31,		As of March 31,
	2016	2017	2018
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 5,357	$ 5,963	$ 57,928
Working capital(1)	4,485	3,683	53,420
Total assets	5,400	7,022	60,216
Warrant to purchase redeemable convertible preferred stock	—	35	47
Derivative liability	88	371	958
Redeemable convertible preferred stock	9,000	26,500	87,500
Total stockholders' deficit	(4,579)	(23,135)	(33,511)

(1) We define working capital as current assets less current liabilities.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks, uncertainties and assumptions. You should read the "Special Note Regarding Forward-Looking Statements" and "Risk Factors" sections of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a Phase 2 clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral-based gene therapies to treat rare diseases following a single dose. Our gene therapies employ hematopoietic stem cells that are extracted from the patient and then modified with lentiviral vectors to insert a functional copy of the gene that is defective in the target disease. We believe that our approach has the potential to provide curative benefit in an outpatient setting for a range of diseases. Our initial focus is on a group of rare genetic diseases referred to as lysosomal storage diseases, which today are primarily managed with enzyme replacement therapies, or ERTs.

We are initially targeting rare diseases in which current standard of care provides the mechanistic proof that the enzymes or proteins produced endogenously following treatment with our gene therapies can offer benefit to patients. Typically in lysosomal storage diseases, a gene mutation results in the deficiency or malfunctioning of an enzyme or other protein. This results in the inability of lysosomes to properly process cellular byproducts. As a result, these byproducts accumulate to toxic levels in the body's cells and, in turn, disrupt the function of multiple tissues and organs. Fabry disease, Gaucher disease and Pompe disease are primarily managed by bi-weekly, multi-hour infusions with ERTs that seek to exogenously replace the missing enzyme. However, given the characteristics of most ERTs, they typically only remain in the plasma for a short period of time and thus, are not ideal because they are only dosed every two weeks. These existing therapies manage, rather than cure, the underlying diseases and, as a result, patients continue to have disease progression. Further, the frequent, periodic and life-long dosing schedule required for ERTs results in significant costs for the healthcare system and is burdensome for the patient.

We seek to develop promising gene therapy programs by applying our expertise in gene and cellular therapies and clinical and regulatory strategy and execution to efficiently bring these potentially curative therapies to patients. In our initial programs, we leverage years of extensive preclinical and early clinical research by leading researchers, as well as our internal research efforts, to advance potential therapies. We plan to identify and develop future product candidates through our own internal research efforts as well as through collaborations with leading academics.

Since our inception in 2015, we have devoted substantially all of our resources to organizing and staffing our company, business planning, raising capital, acquiring or discovering product candidates and securing related intellectual property rights, conducting discovery, research and development activities for our programs and planning for potential commercialization. We do not have any products approved for sale and have not generated any revenue from product sales. To date, we have funded our operations with proceeds from the sales of preferred stock. Through March 31, 2018, we had received gross proceeds of $87.5 million from the sales of our preferred stock. Since our inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our current or future product candidates and programs. Our net losses were $4.7 million and $18.6 million for the years ended December 31, 2016 and 2017, respectively, and

$8.2 million for the three months ended March 31, 2018. As of March 31, 2018 we had an accumulated deficit of $33.6 million. We expect to continue to incur significant expenses for at least the next several years as we advance our product candidates from discovery through preclinical development and clinical trials and seek regulatory approval of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. We may also incur expenses in connection with the in-licensing or acquisition of additional product candidates. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations with proceeds from outside sources, with a majority of such proceeds to be derived from sales of equity, including the anticipated net proceeds from this offering. We also plan to pursue additional funding from outside sources, including our expansion of, or our entry into, new borrowing arrangements; research and development incentive payments from the Australian government; and our entry into potential future collaboration agreements for one or more of our programs. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of March 31, 2018, we had cash and cash equivalents of $57.9 million. We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements into 2020. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "— Liquidity and Capital Resources."

Without giving effect to the anticipated net proceeds from this offering, we expect that our existing cash and cash equivalents will be sufficient to fund our operating expenses and capital expenditure requirements until at least the middle of 2019. To finance our operations beyond that point, we will need to raise additional capital, which cannot be assured.

Components of Our Consolidated Results of Operations

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the near future. If development efforts for our product candidates are successful and result in regulatory approval or additional license agreements with third parties, we may generate revenue in the future from product sales.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred. These expenses include:

- license maintenance fees and milestone fees incurred in connection with various license agreements;
- expenses incurred under agreements with contract research organizations, or CROs, contract manufacturing organizations, or CMOs, as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;
- manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical and clinical trial materials and commercial materials, including manufacturing validation batches;
- employee-related expenses, including salaries, related benefits, travel and stock-based compensation expense for employees engaged in research and development functions;
- costs related to compliance with regulatory requirements; and
- allocated facilities costs, depreciation and other expenses, which include rent and utilities.

We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers.

Our direct research and development expenses are tracked on a program-by-program basis for our product candidates and consist primarily of external costs, such as fees paid to outside consultants, CROs, CMOs, and central laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by program also include fees incurred under license agreements. We do not allocate employee costs or facility expenses, including depreciation or other indirect costs, to specific programs because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to oversee the research and discovery as well as for managing our preclinical development, process development, manufacturing and clinical development activities. These employees work across multiple programs and, therefore, we do not track their costs by program.

The table below summarizes our research and development expenses incurred by program:

	Year Ended December 31,		Three Months Ended March 31,	
	2016	2017	2017	2018
	(in thousands)			
Fabry	$ 105	$ 6,101	$ 387	$1,889
Gaucher	50	2,612	91	855
AML	785	180	166	46
Cystinosis	—	1,030	—	148
Pompe	—	1,010	—	147
Unallocated research and development expenses	1,723	4,258	790	2,562
Total research and development expenses	$2,663	$15,191	$1,434	$5,647

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and development expenses will increase substantially over the next several years, particularly as we increase personnel costs, including stock-based compensation, contractor costs and facilities costs, as we

continue to advance the development of our product candidates. We also expect to incur additional expenses related to milestone and royalty payments payable to third parties with whom we have entered into license agreements to acquire the rights to our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our product candidates or when, if ever, material net cash inflows may commence from any of our product candidates. This uncertainty is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the scope, progress, outcome and costs of our preclinical development activities, clinical trials and other research and development activities;
- establishing an appropriate safety profile with IND-enabling studies;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- development and timely delivery of commercial-grade drug formulations that can be used in our clinical trials and for commercial launch;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- significant and changing government regulation;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

We may never succeed in achieving regulatory approval for any of our product candidates. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on others. Any changes in the outcome of any of these variables with respect to the development of our product candidates in preclinical and clinical development could mean a significant change in the costs and timing associated with the development of these product candidates. For example, if the U.S. Food and Drug Administration, or FDA, or another regulatory authority were to delay our planned start of clinical trials or require us to conduct clinical trials or other testing beyond those that we currently expect, or if we experience significant delays in enrollment in any of our planned clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, related benefits, travel and stock-based compensation expense for personnel in executive, finance and administrative functions. General and administrative expenses also include professional fees for legal, consulting, accounting and audit services.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our product candidates. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, director and officer

insurance costs as well as investor and public relations expenses associated with being a public company. We anticipate the additional costs for these services will substantially increase our general and administrative expenses. Additionally, if and when we believe a regulatory approval of a product candidate appears likely, we anticipate an increase in payroll and expense as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidate.

Other Income (Expense)

Interest Income

Interest income consists of income from bank deposits.

Other Expense

Other expense consists of foreign exchange gain or loss.

Change in Fair Value of Preferred Stock Warrant Liability

In connection with entering into our loan agreement, we agreed to issue a warrant to purchase shares of our preferred stock to the lender. We classify the warrant as a liability on our consolidated balance sheet and we are required to remeasure to fair value at each reporting date. We recognize changes in the fair value of the warrant liability as a component of other income (expense), net in our consolidated statements of operations and comprehensive loss. We will continue to recognize changes in the fair value of the warrant liability until the warrants are exercised, expire or qualify for equity classification.

Change in Fair Value of Derivative Liability

Our stock purchase agreement with University Health Network, or UHN, provides for a payment to UHN upon completion of an initial public offering, or IPO, which includes this offering, if UHN's fully-diluted percentage ownership of our company is reduced within a range of specified percentages. We classify the IPO dilution payment obligation as a liability on our consolidated balance sheet and we are required to remeasure to fair value at each reporting date. We recognize changes in the fair value of the derivative liability as a component of other income (expense), net in our consolidated statements of operations and comprehensive loss. We will continue to recognize changes in the fair value of the derivative liability until an IPO occurs.

Consolidated Results of Operations

Comparison of the Years Ended December 31, 2016 and 2017

The following table summarizes our consolidated results of operations for the years ended December 31, 2016 and 2017:

	Year Ended December 31,		
	2016	2017	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 2,663	$ 15,191	$ 12,528
General and administrative	1,962	3,195	1,233
Total operating expenses	4,625	18,386	13,761
Loss from operations	(4,625)	(18,386)	(13,761)
Other income (expense):			
Interest income	6	57	51
Change in fair value of preferred stock warrant liability	—	(17)	(17)
Change in fair value of derivative liability	(39)	(283)	(244)
Other expense	(6)	(19)	(13)
Total other expense, net	(39)	(262)	(223)
Net loss	$(4,664)	$(18,648)	$(13,984)

Research and Development Expenses

	Year Ended December 31,		
	2016	2017	Change
	(in thousands)		
Direct research and development expenses by program:			
Fabry	$ 105	$ 6,101	$ 5,996
Gaucher	50	2,612	2,562
AML	785	180	(605)
Cystinosis	—	1,030	1,030
Pompe	—	1,010	1,010
Unallocated research and development expenses:			
Personnel related (including stock-based compensation)	1,298	3,203	1,905
Other	425	1,055	630
Total research and development expenses	$2,663	$15,191	$12,528

Research and development expenses were $2.7 million for the year ended December 31, 2016, compared to $15.2 million for the year ended December 31, 2017. The increase of $12.5 million was primarily due to increases of $6.0 million in direct costs for our Fabry program, $2.6 million in direct costs connected with our Gaucher program, $1.0 million in direct costs related to our Cystinosis program, $1.0 million in direct costs connected with our Pompe program, and $2.5 million in research and discovery and unallocated costs, all partially offset by a decrease of $0.6 million in direct costs for our AML program as we shifted our focus onto developing our other programs.

The increase in direct costs for our Fabry program was primarily due to pre-clinical, clinical and process development cost of $3.1 million, as well as CMO, CRO and consulting fees of $2.5 million.

The increase in direct costs for our Gaucher program was primarily due to pre-clinical and process development cost of $1.2 million, as well as CMO fees of $1.0 million.

The increase in direct costs for our Cystinosis program was primarily due to upfront license cost of $1.0 million paid to GenStem.

The increase in direct costs for our Pompe program was primarily due to upfront license cost of $0.5 million paid and $0.5 million payable to BioMarin.

The increase in research and discovery and unallocated costs was primarily due to an increase of $1.9 million in personnel-related costs, including stock-based compensation, as a result of hiring additional personnel in our research and development department and an increase of $0.6 million in unallocated consulting expenses and facility costs and rent expense. Personnel-related costs for the years ended December 31, 2016 and 2017 included stock-based compensation expense of less than $0.1 million.

General and Administrative Expenses

General and administrative expenses were $2.0 million for the year ended December 31, 2016, compared to $3.2 million for the year ended December 31, 2017. The increase of $1.2 million was primarily due to increases of $0.4 million in personnel-related costs, including stock-based compensation, $0.3 million in consulting expense, $0.1 million in professional fees and $0.2 million in facility expense. The increase in personnel-related costs was due to the hiring of additional personnel in our general and administrative functions, including the hiring of our CFO in late 2017. Professional fees increased due to costs associated with the preparation of our financial statements as well as ongoing business operations. The increase in facility expense was primarily due to the addition of increased office space as a result of the continued growth of the employee headcount.

Other Income (Expense), net

Other income (expense), net was not significant during either of the years ended December 31, 2016 or 2017.

Comparison of the Three Months Ended March 31, 2017 and 2018

The following table summarizes our consolidated results of operations for the three months ended March 31, 2017 and 2018:

	Three Months Ended March 31,		
	2017	2018	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 1,434	$ 5,647	$ 4,213
General and administrative	610	2,141	1,531
Total operating expenses	2,044	7,788	5,744
Loss from operations	(2,044)	(7,788)	(5,744)
Other income (expense):			
Interest income	4	158	154
Change in fair value of preferred stock warrant liability	—	(12)	(12)
Change in fair value of derivative liability	(32)	(587)	(555)
Other expenses	(5)	(13)	(8)
Total other expense, net	(33)	(454)	(421)
Net loss	$(2,077)	$(8,242)	$(6,165)

Research and Development Expenses

	Three Months Ended March 31, 2017	2018	Change
	(in thousands)		
Direct research and development expenses by program:			
Fabry	$ 387	$1,889	$1,502
Gaucher	91	855	764
AML	166	46	(120)
Cystinosis	—	148	148
Pompe	—	147	147
Unallocated research and development expenses:			
Personnel related (including stock-based compensation)	636	1,621	985
Other	154	941	787
Total research and development expenses	$1,434	$5,647	$4,213

Research and development expenses were $1.4 million for the three months ended March 31, 2017, compared to $5.6 million for the three months ended March 31, 2018. The increase of $4.2 million was primarily due to increases of $1.5 million in direct costs for our Fabry program, $0.8 million in direct costs connected with our Gaucher program, $0.1 million in direct costs related to our Cystinosis program, $0.1 million in direct costs related to our Pompe program, and $1.6 million in research and discovery and unallocated costs, all partially offset by a decrease of $0.1 million in direct costs for our AML program as we shifted our focus onto developing our other programs.

The increase in direct costs for our Fabry program was primarily due to pre-clinical, clinical and process development cost of $1.1 million, as well as CMO, CRO and consulting fees of $0.4 million.

The increase in direct costs for our Gaucher program was primarily due to pre-clinical and process development cost of $0.8 million.

The increase in direct costs for our Cystinosis program was primarily due to consulting fees of $0.1 million.

The increase in direct costs for our Pompe program was primarily due to development costs of $0.1 million.

The increase in research and discovery and unallocated costs was primarily due to an increase of $1.0 million in personnel-related costs, including stock-based compensation, as a result of hiring additional personnel in our research and development department and an increase of $0.8 million in unallocated consulting expenses and facility costs and rent expense.

General and Administrative Expenses

General and administrative expenses were $0.6 million for the three months ended March 31, 2017, compared to $2.1 million for the three months ended March 31, 2018. The increase of $1.5 million was primarily due to increases of $0.6 million in personnel-related costs, including stock-based compensation, $0.6 million in consulting expense, $0.2 million in professional fees and $0.1 million in legal expense. The increase in personnel-related costs was due to the hiring of additional personnel in our general and administrative functions, including the hiring of our CFO in late 2017. Professional fees increased due to costs associated with the preparation of our consolidated financial statements as well as ongoing business operations.

Other Income (Expense), net

Other income (expense), net was a loss of less than $0.1 million for the three months ended March 31, 2017, compared to a loss of $0.5 million for the three months ended March 31, 2018. The increase in other expense of $0.4 million was primarily due to increase of $0.6 million in change fair value of derivative liability, partially offset by a $0.2 million increase in interest income.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue and have incurred significant operating losses and negative cash flows from our operations. We have funded our operations to date primarily with proceeds from the sale of preferred stock. Through March 31, 2018, we had received gross cash proceeds of $87.5 million from sales of our preferred stock.

Cash in excess of immediate requirements is invested primarily with a view to liquidity and capital preservation.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

	Year Ended December 31,		Three Months Ended March 31,	
	2016	2017	2017	2018
	(in thousands)			
Net cash used in operating activities	$(3,314)	$(16,382)	$(2,011)	$ (5,980)
Net cash used in investing activities	(24)	(383)	(55)	(263)
Net cash provided by financing activities	8,695	17,371	3,487	58,208
Net increase in cash and cash equivalents	$ 5,357	$ 606	$ 1,421	$51,965

Operating Activities

During the three months ended March 31, 2018, operating activities used $6.0 million of cash and cash equivalents, resulting from our net loss of $8.2 million, partially offset by non-cash charges of $0.9 million and net cash provided by changes in our operating assets and liabilities of $1.3 million. The net changes in our operating assets and liabilities was primarily due to increases in liabilities of $1.8 million due to ongoing research, development, and clinical trial efforts and partially offset by increases in assets of $0.5 million, including an increase due to a $0.2 million security deposit for a new lease that was executed in 2018.

During the three months ended March 31, 2017, operating activities used $2.0 million of cash and cash equivalents, resulting from our net loss of $2.1 million, primarily offset by non-cash charges of $0.2 million and net cash used by changes in our operating assets and liabilities of $0.1 million. Net cash used by changes in our operating assets and liabilities for the three months ended March 31, 2017 consisted primarily of a $0.1 million increase in prepaid expenses and other current assets.

During the year ended December 31, 2017, operating activities used $16.4 million of cash and cash equivalents, resulting from our net loss of $18.6 million, partially offset by non-cash charges of $0.7 million and net cash provided by changes in our operating assets and liabilities of $1.6 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2017 consisted primarily of a $1.5 million increase in accrued expenses and other current liabilities, a $0.5 million increase in other long-term liability and a $0.2 million increase in accounts payable, partially offset by a $0.2 million increase in other assets and a $0.3 million increase in prepaid expenses and other current assets. The increases in accrued expenses and other

current liabilities were primarily due to ongoing research, development, and clinical trial work, and an increase in the incentive bonus accrual as of December 31, 2017. The increase in prepaid expenses and other current assets was primarily due to a $0.1 million increase in prepaid development costs associated with our Gaucher program and a $0.1 million increase in prepaid rent upon commencement of two new leases during 2017.

During the year ended December 31, 2016, operating activities used $3.3 million of cash and cash equivalents, resulting from our net loss of $4.7 million, primarily offset by non-cash charges of $0.6 million and net cash provided by changes in our operating assets and liabilities of $0.8 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2016 consisted primarily of a $0.6 million increase in accrued expenses and other current liabilities and $0.2 million increase in accounts payable. The increase in accrued expenses and other current liabilities was due to an increase in professional fees and personnel costs associated with establishing our Cambridge, Massachusetts operations.

Investing Activities

During the three months ended March 31, 2018, we used $0.3 million of cash and cash equivalents in investing activities consisting of purchases of property and equipment. During the three months ended March 31, 2017, we used less than $0.1 million of cash and cash equivalents in purchases of property and equipment.

During the year ended December 31, 2017, we used $0.4 million of cash and cash equivalents in investing activities consisting of purchases of property and equipment. During the year ended December 31, 2016, we used an insignificant amount of cash and cash equivalents in investing activities consisting of payment of a leasing deposit.

Financing Activities

During the three months ended March 31, 2018, net cash provided by financing activities was $58.2 million, primarily consisting of net cash proceeds from our issuance of Series B preferred stock in January 2018.

During the three months ended March 31, 2017, net cash provided by financing activities was $3.5 million, consisting of net cash proceeds from our issuance of Series A preferred stock in March 2017.

During the year ended December 31, 2017, net cash provided by financing activities was $17.4 million, primarily consisting of net cash proceeds of $17.4 million from our issuance of Series A preferred stock in March 2017 and October 2017.

During the year ended December 31, 2016, net cash provided by financing activities was $8.7 million, primarily consisting of net cash proceeds of $1.4 million from our issuance of Series Seed preferred stock in January 2016 and net proceeds of $7.3 million from our issuance of Series A preferred stock in July 2016.

Term Loan Agreement

In June 2017, we entered into a Loan and Security Agreement, which we refer to as the Loan Agreement, with Silicon Valley Bank, or SVB, providing a senior secured non-revolving loan facility of up to an aggregate principal amount of $10.0 million, available for us to draw down in three tranches until October 31, 2018, subject to the satisfaction of certain milestones for each tranche. As of March 31, 2018, we had not drawn down from the facility and the $3.5 million first tranche was available.

The first tranche of $3.5 million was made available upon entry into the Loan Agreement as we satisfied the borrowing conditions at such time. The second tranche of $3.5 million will be made available after the funding of the first tranche amounts and upon confirmation by SVB that either we have met certain clinical and developmental milestones, or we have subsequently obtained at least $7.5 million in cash proceeds from the sale

of our equity securities from investors reasonably acceptable to SVB. The third tranche of $3.0 million will be made available after the funding of the first and second tranche amounts and upon confirmation by SVB that we have subsequently obtained at least an additional $6.5 million in cash proceeds from the sale of our equity securities to investors reasonably acceptable to SVB, and we have received a signed and enforceable term sheet from investors acceptable to SVB committing to provide financing on or before March 31, 2018 in an amount equal to at least 12 months of operating expenses. In January 2018, we received gross cash proceeds of $60.5 million from the sale of our Series B preferred stock.

Any outstanding principal amounts under the Loan Agreement will accrue interest at a floating per annum rate equal to the greater of 1% and the "prime rate," as published in the Wall Street Journal, minus 3%. Payments on the Loan Agreement are interest only, payable monthly in arrears, until November 1, 2018, which can be extended by six months if the third tranche is drawn. Thereafter, principal and interest amounts are repayable over a 30-month period, unless the third tranche is funded and the initial interest-only period is extended by six months, in which case principal and interest amounts are repayable over a 24-month period.

Pursuant to the Loan Agreement, we provided a first priority security interest in all existing and after-acquired assets, excluding intellectual property and certain other assets owned by us. The Loan Agreement contains a negative pledge on our intellectual property.

In connection with the Loan Agreement, we issued a warrant to SVB to purchase shares of our Series A preferred stock at an exercise price of $0.7949 per share. This warrant is initially exercisable for 28,305 shares of Series A preferred stock. Up to an additional 160,397 shares of Series A preferred stock may become subject to this warrant, with the proportion of such additional shares equal to the percentage of the full $10.0 million aggregate principal amount under the Loan Agreement that we draw down thereunder.

The Loan Agreement allows us to voluntarily prepay all but not less than all the outstanding amounts thereunder. A scaling prepayment fee of 1% or 0.5% would be assessed if we prepay the amounts within the first anniversary of funding, or between the first and second anniversary of funding, respectively. No prepayment fee would be assessed if we prepay the amounts after the second anniversary of funding. A final payment fee of 6.75% multiplied by the original principal amount of each tranche drawn is due upon the earliest to occur of the maturity date of the Loan Agreement, the termination of the Loan Agreement, the acceleration of the Loan Agreement or repayment or prepayment of such borrowings.

The Loan Agreement contains customary indemnification obligations and customary events of default, including, among other things, our failure to fulfill certain of our obligations under the Loan Agreement and the occurrence of a material adverse change in our business, operations or condition, a material impairment of the prospect of repayment of any portion of the loan, or a material impairment in the perfection or priority of the SVB's lien in the collateral or in the value of such collateral. In the event of default by us under the Loan Agreement, SVB would be entitled to exercise its remedies thereunder, including the right to accelerate the debt, upon which we may be required to repay all amounts then outstanding under the Loan Agreement or SVB may take possession of the collateral securing the Loan Agreement.

The Loan Agreement includes certain restrictions on, among other things, our ability to incur additional indebtedness, change the name or location of our business, merge with or acquire other entities, pay dividends or make other distributions to holders of our capital stock, make certain investments, engage in transactions with affiliates, create liens, open new deposit accounts, sell assets or pay subordinated debt.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials of our product candidates. In addition, upon the closing of

this offering, we expect to incur additional costs associated with operating as a public company. Our expenses will also increase as we:

- continue our development of our product candidates, including continuing enrollment in our recently initiated Phase 2 clinical trial for AVR-RD-01;
- initiate additional clinical trials and preclinical studies for our other product candidates;
- seek to identify and develop or in-license or acquire additional product candidates and technologies;
- seek to industrialize our *ex vivo* lentiviral gene therapy approach into a robust, scalable and, if approved, commercially viable process;
- seek marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- hire and retain additional personnel, such as clinical, quality control, commercial and scientific personnel;
- expand our infrastructure and facilities to accommodate our growing employee base, including adding equipment and physical infrastructure to support our research and development; and
- transition our organization to being a public company.

We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements into 2020. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. If we receive regulatory approval for AVR-RD-01 or our other product candidates, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution, depending on where we choose to commercialize.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaboration agreements, government and other third-party funding, strategic alliances, licensing arrangements or marketing and distribution arrangements. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through government and other third-party funding, collaboration agreements, strategic alliances, licensing arrangements or marketing and distribution arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2017 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments Due by Period				
	Total	Less than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
	(in thousands)				
Operating lease commitments	$1,641	$ 395	$682	$341	$ 223
Total	$1,641	$ 395	$682	$341	$ 223

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for clinical trials, preclinical research studies and testing and manufacturing services. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation. These payments are not included in the preceding table as the amount and timing of such payments are not known.

Additionally, the table above excludes the payment that may be due to UHN upon the closing of the sale of shares of common stock in an IPO, which includes this offering. If UHN's fully-diluted percentage ownership of our company is reduced within a range of specified percentages in an IPO, then we are obligated to pay UHN an amount up to $2.0 million. We have not included the UHN dilution payment in the preceding table as the amount, timing and likelihood of such payments are not known.

In addition, pursuant to our license agreements with UHN, BioMarin, GenStem and the Lund University rights holders, we are required to make certain milestone and royalty payments to our licensors. See "Business—License Agreements" for additional details regarding our payment obligations to these licensors.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors, including central laboratories, in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical and clinical studies; and
- CMOs in connection with drug substance and drug product formulation of preclinical and clinical trial materials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that

conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We measure stock options and other stock-based awards granted to employees and members of our board of directors for their services as directors based on the fair value on the date of the grant and recognize the corresponding compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. We have only issued stock options with service-based vesting conditions and record the expense for these awards using the straight-line method.

For stock-based awards granted to consultants and non-employees, we recognize compensation expense over the period during which services are rendered by such consultants and non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of our common stock and updated assumption inputs in the Black-Scholes option-pricing model.

We estimate the fair value of each stock option grant using the Black-Scholes option-pricing model, which uses as inputs the estimated fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of our stock options, the risk-free interest rate for a period that approximates the expected term of our stock options and our expected dividend yield.

We determined the assumptions for the Black-Scholes option-pricing model as discussed below. Each of these inputs is subjective and generally requires significant judgment to determine.

- *Fair Value of Our Common Stock.* Prior to this offering, our stock was not publicly traded, and therefore we estimated the fair value of our common stock, as discussed in “Determination of the Fair Value of Common Stock” below.
- *Expected Term.* The expected term represents the period that the stock-based awards are expected to be outstanding. The expected term of stock options granted has been determined using the simplified method, which uses the midpoint between the vesting date and the contractual term.
- *Risk-Free Interest Rate.* The risk-free interest rate is based on the U.S. Treasury yield curve in effect at the date of grant for zero-coupon U.S. Treasury constant maturity notes with terms approximately equal to the stock-based award’s expected term.
- *Expected Volatility.* Because we do not have a trading history of our common stock, the expected volatility was derived from the average historical stock volatilities of several public companies within our industry that we consider to be comparable to our business over a period equivalent to the expected term of the stock-based awards.
- *Dividend Rate.* The expected dividend is zero as we have not paid and do not anticipate paying any dividends in the foreseeable future.

If any of the assumptions used in the Black-Scholes model change significantly, stock-based compensation for future awards may differ materially compared with the awards granted previously.

The weighted-average fair values of options granted during the years ended December 31, 2016 and 2017 were $0.50 and $1.50, respectively. No options were granted during the three months ended March 31, 2017. The weighted-average fair value of options granted during the three months ended March 31, 2018 was $6.99. The weighted-average assumptions utilized to determine the fair value of options granted are presented in the following table:

	Year Ended December 31,		Three Months Ended March 31, 2018
	2016	2017	
Expected option life (years)	6.00	6.08	6.07
Risk-free interest rate	1.39%	1.93%	2.72%
Expected volatility	86.00%	84.54%	84.00%
Expected dividend yield	—%	—%	—%

Stock-based Awards Granted

The following table sets forth by grant date the number of shares subject to options granted since January 1, 2017, the per share exercise price of the options, the fair value of common stock per share on each grant date, and the per share estimated fair value of the options:

Grant Date	Number of Shares Subject to Options Granted	Per Share Exercise Price of Options	Fair Value per Common Share on Grant Date
June 13, 2017	192,839	$ 0.91	$ 0.91
June 26, 2017	4,033	$ 0.91	$ 0.91
July 10, 2017	8,524	$ 0.91	$ 0.91
July 17, 2017	12,178	$ 0.91	$ 0.91
August 28, 2017(1)	158,318	$ 0.91	$ 2.19
October 4, 2017(2)	8,470	$ 0.91	$ 4.09
October 17, 2017(2)	2,420	$ 0.91	$ 4.09
October 24, 2017(2)	48,740	$ 0.91	$ 4.09
March 16, 2018(3)	753,789	$ 5.00	$ 8.76

(1) At the time of the option grants in August 2017, our board of directors determined that the fair value of our common stock of $0.91 per share calculated in the contemporaneous valuation as of March 31, 2017 reasonably reflected the per share fair value of one share of our common stock as of the grant date. However, as described below, the fair value of our common stock at this date was adjusted to $2.19 per share in connection with a retrospective fair value assessment for financial reporting purposes.

(2) At the time of the option grants in October 2017, our board of directors determined that the fair value of our common stock of $0.91 per share calculated in the contemporaneous valuation as of March 31, 2017 reasonably reflected the per share fair value of one share of our common stock as of the grant date. However, as described below, the fair value of our common stock at the date of these grants was adjusted to $4.09 per share in connection with a retrospective fair value assessment for financial reporting purposes.

(3) At the time of the option grants in March 2018, our board of directors determined that the fair value of our common stock of $5.00 per share calculated in the contemporaneous valuation as of January 31, 2018 reasonably reflected the per share fair value of one share of our common stock as of the grant date. However, in connection with the preparation of the Company's unaudited condensed consolidated financial statements for the three months ended March 31, 2018, the fair value of our common stock at the date of these grants was adjusted to $6.03 per share as a result of a contemporaneous valuation performed as of March 31, 2018. In connection with determining the estimated price range for this offering, based on the midpoint of $17.00 per share on the cover of our preliminary prospectus filed with the Securities and Exchange Commission on June 18, 2018, we re-assessed the fair value of our common stock for financial reporting purposes through a retrospective valuation as of March 31, 2018, resulting in a further adjustment of the fair value of our common stock as of such date to $8.76. Such adjustment results in additional stock-based compensation expense of approximately $1.8 million which will be recognized over the four-year vesting period for these awards. The effect of this adjustment did not have a material effect on the interim financial statements for the three-month period ended March 31, 2018.

Determination of the Fair Value of Common Stock

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant, with input from management, considering third-party valuations of our common stock as well as our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent third-party valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, Valuation of Privately-Held-Company Equity Securities Issued as Compensation. Once a public trading market for our common stock has been established following the closing of this offering, it will no longer be necessary for our board of directors to estimate the fair market value of our common stock in connection with our accounting for granted equity awards.

For financial reporting purposes, we performed ordinary share valuations, with the assistance of a third-party specialist, at various dates, which resulted in valuations of our common stock of $0.41 per share as of January 31, 2016, $1.20 per share as of August 31, 2016, $0.91 per share as of March 31, 2017, $2.19 per share as of August 31, 2017, $4.09 per share as of October 31, 2017, $5.00 per share as of January 31, 2018, and $8.76 as of March 31, 2018. In conducting the valuations, our board of directors, with input from management, considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, including:

- the prices at which we sold preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;
- the progress of our research and development programs, including the status of preclinical studies and planned clinical trials for our product candidates;
- our stage of development and commercialization and our business strategy;
- external market conditions affecting the biotechnology industry, and trends within the biotechnology industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock and our preferred stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or a sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

As described above, for March 31, 2018, in connection with determining the estimated price range for this offering, based on the midpoint of $17.00 per share on the cover of our preliminary prospectus filed with the Securities and Exchange Commission on June 18, 2018, we re-assessed the fair value of our common stock for financial reporting purposes through a retrospective valuation that resulted in an adjustment of the fair value of our common stock as of such date to $8.76. Such adjustment results in additional stock-based compensation expense of approximately $1.8 million which will be recognized over the four-year vesting period for these awards. The effect of this adjustment did not have a material effect on the interim financial statements for the three-month period ended March 31, 2018.

The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

The dates of our valuations have not always coincided with the dates of our stock option grants. In determining the fair value of the shares underlying options set forth in the table above, we considered, among other things, the most recent contemporaneous valuations of our ordinary shares and our assessment of additional objective and subjective factors we believed were relevant as of the grant date. The additional factors considered

when determining any changes in fair value between the most recent contemporaneous valuation and the grant dates included our stage of development and commercialization and our business strategy, our operating and financial performance and current business conditions.

Following the closing of this offering, the fair value of our common stock will be determined based on the quoted market price of our common stock.

Our common stock valuations were prepared using the option-pricing method, or OPM, which treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. The future value of the common stock is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock.

Beginning with the August 23, 2017 valuation, we changed the methodology for allocating our equity value to our common stock to a hybrid method, which is a combination of a probability weighted expected return method, or PWERM and an OPM. We made this change as greater certainty developed regarding a possible liquidity event. The PWERM methodology relies on a forward-looking analysis to predict the possible future value of a company. Under this method, discrete future outcomes, such as an IPO, non-IPO scenarios, and a merger or sale are weighted based on our estimate of the probability of each scenario. In our application of the hybrid method, we considered an IPO scenario under the PWERM framework, and a non-IPO scenario modeled using an OPM to reflect the full distribution of possible non-IPO outcomes. The hybrid method is useful when certain discrete future outcomes can be predicted, but also accounts for uncertainty regarding the timing or likelihood of specific alternative exit events.

Determination of Initial Public Offering Price

We and our underwriters determined the initial public offering price per share of $19.00. In comparison, our estimate of the fair value of our common stock was $0.91 per share at June 13, 2017, June 26, 2017, July 10, 2017 and July 17, 2017, which was determined by our board of directors with the assistance of a third-party valuation of our common stock as of March 31, 2017. Our estimate of the fair value of our common stock as of August 28, 2017 was retrospectively adjusted with the assistance of a third-party valuation of our common stock as of August 31, 2017. Our estimate of the fair value of our common stock as of October 4, 2017, October 17, 2017 and October 24, 2017 was retrospectively adjusted to $4.09 with the assistance of a third-party valuation of our common stock as of October 24, 2017. Our estimate of the fair value of our common stock was $5.00 at March 16, 2018, which was determined by our board of directors with the assistance of a third-party valuation of our common stock as of January 31, 2018, subsequently retrospectively adjusted to $8.76 with the assistance of a third-party valuation of our common stock as of March 31, 2018.

These valuations utilized the hybrid method described in "—Determination of the Fair Value of Common Stock." The valuation for our June 13, 2017, June 26, 2017, July 10, 2017 and July 17, 2017 stock option grants did not consider an initial public offering, or IPO, scenario, and reflected a discount for lack of marketability of 36%. The valuation used for the retrospective fair value assessment of our August 28, 2017 option grants attributed a 10% probability to an IPO scenario and a 90% probability of a non-IPO scenario and reflected a discount for lack of marketability of 20% and 30% to the IPO and non-IPO scenario, respectively. The valuation used for the retrospective fair value assessment of our October 4, 2017, October 17, 2017 and October 24, 2017 option grants attributed a 15% probability to an IPO scenario and a 85% probability of a non-IPO scenario and reflected a discount for lack of marketability of 17% and 25% to the IPO and non-IPO scenario, respectively. The valuation used for the retrospective fair value assessment of our March 16, 2018 option grants attributed a 50% probability to an IPO scenario and a 50% probability of a non-IPO scenario and reflected a discount for lack of marketability of 12% and 25% to the IPO and non-IPO scenario, respectively. In addition to quantitative analysis from third-party valuations of our common stock, we also considered macro-economic and market conditions,

including our subjective assessment of market conditions for initial public offerings of companies similarly situated to ours and our subjective assessment as to the likelihood of successfully executing an initial public offering in the coming months, among other factors.

We note that, as is typical in initial public offerings, the initial public offering price per share for this offering was not derived using a formal determination of fair value, but was determined based upon discussions between us and the underwriters. Among the factors considered in setting the initial public offering price per share were prevailing market conditions, estimates of our business potential, progress in our research and development programs and developments in our business, the general condition of the securities market and the market prices of, and demand for, publicly-traded common stock of generally comparable companies.

Valuation of Derivative Liability

The fair value of the derivative liability recognized in connection with our stock purchase agreement with UHN was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the derivative liability was determined using the PWERM, which considered as inputs the type and probability of occurrence of an IPO dilution event, the amount of the payment, the expected timing of an IPO dilution event and a risk-adjusted discount rate.

Valuation of Warrant Liability

In connection with entering into a loan agreement, we agreed to issue a warrant to purchase shares of our Series A preferred stock to the lender. We classify the warrant as a liability on our consolidated balance sheet because the warrant represents a free-standing financial instrument that may require us to transfer assets upon exercise. The warrant liability was initially recorded at fair value upon the date of the warrant issuance and is subsequently remeasured to fair value at each reporting date. Changes in the fair value of the warrant liability are recognized as a component of other income (expense), net in the consolidated statements of operations and will continue to be recognized until the warrant is exercised, expires or qualifies for equity classification.

We utilize the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value the warrant. We assess these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying equity instruments issuable upon exercise of the warrant, the remaining contractual term of the warrant, risk-free interest rate, expected dividend yield and expected volatility of the underlying preferred stock. We have historically been a private company and lack company-specific historical and implied volatility information of our stock. Therefore, we estimate expected stock volatility based on the historical volatility of publicly traded peer companies for a term equal to the remaining contractual term of the warrant. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrant. We have estimated a 0% dividend yield based on the fact that we have never paid or declared dividends.

Upon the closing of this offering, the preferred stock warrant will become exercisable for common stock instead of preferred stock, and the remeasured fair value of the warrant liability will be reclassified to additional paid-in capital.

Quantitative and Qualitative Disclosures about Market Risks

Interest Rate Risk

As of March 31, 2018, we had cash and cash equivalents of $57.9 million, which consisted of cash and money market funds. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in interest rates would not have a material impact on our cash and cash equivalents, financial position or results of operations.

Foreign Currency Exchange Risk

We are exposed to foreign exchange rate risk. Our headquarters are located in the United States, where the majority of our general and administrative expenses and research and development costs are incurred in U.S. dollars. A portion of our research and development costs are incurred by our subsidiaries in Australia and Canada, whose functional currencies are the U.S. dollar but engage in transactions in Australian dollars and Canadian dollars, respectively. During each of the years ended December 31, 2016 and 2017, we recognized foreign currency transaction losses of $6,000 and $19,000, respectively. During each of the three months ended March 31, 2017 and 2018, we recognized foreign currency transaction losses of $5,000 and $13,000, respectively. These losses primarily related to unrealized and realized foreign currency gains and losses as a result of transactions entered into by our Australian and Canadian subsidiaries in currencies other than the U.S. dollar. These foreign currency transaction gains and losses were recorded in other expense, net in our consolidated statements of operations. We believe that a 10% change in the exchange rate between the U.S. dollar, Australian dollar and Canadian dollar would not have a material impact on our financial position or results of operations.

As we continue to grow our business, our results of operations and cash flows will be subject to fluctuations due to changes in foreign currency exchange rates, which could adversely impact our results of operations. To date, we have not entered into any foreign currency hedging contracts to mitigate our exposure to foreign currency exchange risk.

Emerging Growth Company Status

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. We may take advantage of these exemptions until we are no longer an emerging growth company. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. We have elected to use the extended transition period for complying with new or revised accounting standards and, as a result of this election, our financial statements may not be comparable to companies that comply with public company effective dates. We may take advantage of these exemptions up until the last day of the fiscal year following the fifth anniversary of this offering or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1.07 billion in annual revenue, we have more than $700.0 million in market value of our stock held by non-affiliates (and we have been a public company for at least 12 months and have filed one annual report on Form 10-K) or we issue more than $1.0 billion of non-convertible debt securities over a three-year period.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 15 to our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus.

BUSINESS

Overview

We are a Phase 2 clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral-based gene therapies to treat rare diseases following a single dose. Our gene therapies employ hematopoietic stem cells that are extracted from the patient and then modified with lentiviral vectors to insert a functional copy of the gene that is defective in the target disease. We believe that our approach has the potential to provide curative benefit in an outpatient setting for a range of diseases. Our initial focus is on a group of rare genetic diseases referred to as lysosomal storage diseases, which today are primarily managed with enzyme replacement therapies, or ERTs. These lysosomal storage diseases have well understood biologies, identified patient populations and represent large market opportunities with approximately $4.0 billion in worldwide net sales in 2017.

Our initial pipeline is comprised of four lentiviral-based gene therapies, including AVR-RD-01 for the treatment of Fabry disease, AVR-RD-02 for the treatment of Gaucher disease, AVR-RD-03 for the treatment of Pompe disease and AVR-RD-04 for the treatment of cystinosis. AVR-RD-01 is currently being evaluated in an investigator-sponsored Phase 1 clinical trial and has demonstrated clinically significant increased enzyme activity to date, with plasma a-galactosidase A, or AGA, enzyme activity levels in both patients treated to date increasing above the range for males with classic Fabry disease, defined as less than 1 nmol/hr/ml. On June 7, 2018, we dosed the first patient in our company-sponsored Phase 2 clinical trial of AVR-RD-01. In addition, Phase 1/2 clinical trials for both AVR-RD-02 and AVR-RD-04 are being planned and we expect patients will be dosed in 2019.

Lentiviral-based gene therapy has been observed to be well-tolerated in third parties' ongoing clinical trials for rare diseases such as beta thalassemia, ALD and ADA-SCID. To date, over 200 patients have been treated with lentiviral-based gene therapies in third parties' clinical trials. Historically, the use of *ex vivo* lentiviral-based therapies has been restricted primarily to the most acutely severe diseases where risks of the typical requirement for heavily ablating the patients' bone marrow and thus significantly impairing these patients' immune systems had an acceptable risk/benefit profile. The ablation procedure, also known as the conditioning regimen, is administered prior to the gene therapy. The more intensive the conditioning regimen, the greater the risk of toxicity and thus the need for more intensive in-patient monitoring and potential for lengthy hospitalization.

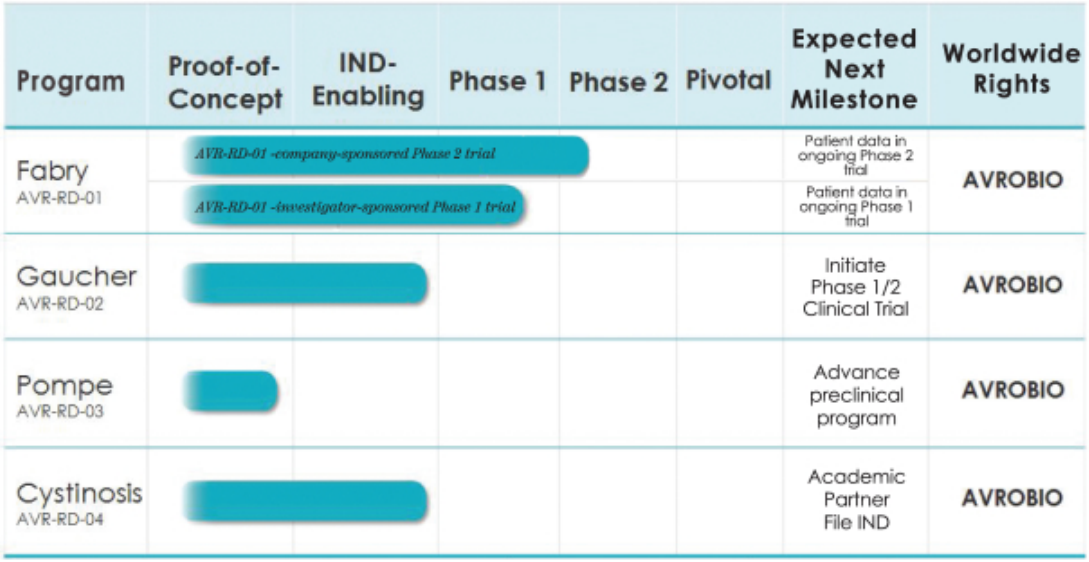
Our goal is to broaden the applicability of lentiviral-based gene therapy by initially targeting diseases where we generally believe durable effects can be achieved following a milder conditioning regimen that allows for outpatient treatment. We believe our approach of choosing diseases where the conditioning regimen can be milder, thus improving patient tolerability, will extend the reach of our gene therapies to a broad range of diseases as first-line therapies.

We are initially targeting rare diseases in which current standard of care provides the mechanistic proof that the enzymes or proteins produced endogenously following treatment with our gene therapies can offer benefit to patients. Typically in lysosomal storage diseases, a gene mutation results in the deficiency or malfunctioning of an enzyme or other protein. This results in the inability of lysosomes to properly process cellular byproducts. As a result, these byproducts accumulate to toxic levels in the body's cells and, in turn, disrupt the function of multiple tissues and organs. Fabry disease, Gaucher disease and Pompe disease are primarily managed by bi-weekly, multi-hour infusions with ERTs that seek to exogenously replace the missing enzyme. However, given the characteristics of most ERTs, they typically only remain in the plasma for a short period of time and thus, are not ideal because they are only dosed every two weeks. These existing therapies manage, rather than cure, the underlying diseases and, as a result, patients continue to have disease progression. Further, the frequent, periodic and life-long dosing schedule required for ERTs results in significant costs for the healthcare system and is burdensome for the patient.

We believe our gene therapies leverage the well understood mechanism of ERTs by transforming a patient's own cells into a drug product that enables the patient to express functional enzyme or other protein and mirror

the biology seen in an otherwise healthy individual. We believe that a single dose of our gene therapies may provide meaningful life-long benefit to these patients and potentially cure these diseases while also providing significant health economic advantages.

Our programs leverage years of extensive preclinical and early clinical research by leading researchers, as well as our internal research efforts. The status of our initial lentiviral-based gene therapy programs is reflected below.



Our lead product candidate, AVR-RD-01 for the treatment of Fabry disease, is derived from hematopoietic stem cells to which the gene encoding AGA is added in an *ex vivo* process using a lentiviral vector. In an ongoing Phase 1 clinical trial of patients with Fabry disease, AVR-RD-01 has been well-tolerated and has led to the production of active AGA enzyme in the two patients treated to date. The first patient dosed in this trial continues to express plasma activity levels of AGA enzyme above the range for males with classic Fabry disease twelve months after receiving AVR-RD-01. Plasma AGA enzyme activity levels in the second patient also began increasing after receiving AVR-RD-01 and remained above the range for males with classic Fabry disease three months after treatment. In addition, we have initiated our company-sponsored Phase 2 clinical trial of AVR-RD-01 and recently dosed the first patient in this trial on June 7, 2018.

Preclinical data for both our Gaucher and cystinosis programs have demonstrated positive results and we expect to begin dosing patients in Phase 1/2 clinical trials for both AVR-RD-02 (Gaucher) and AVR-RD-04 (cystinosis) in 2019. AVR-RD-03 for Pompe disease is currently in early preclinical development. We continue to seek opportunities to expand our approach to other rare and non-rare diseases. We plan to identify and develop future product candidates through our own internal research efforts as well as through collaborations with leading researchers worldwide.

We have developed a detailed plan for the more cost efficient and scalable manufacturing of our product candidates. We are establishing global manufacturing capabilities to support all aspects of the development and, if approved, the eventual commercialization of our gene therapies, from lentiviral vector production to cell processing. We are currently executing on our plans to move to a closed, automated manufacturing system, which we expect to complete by the end of 2019. We also utilize a cryopreservation process that we believe will allow for the global distribution and, if approved, commercialization of our gene therapies.

Our Expertise

We are led by biopharmaceutical experts with extensive experience in gene and cellular therapy and rare diseases. Our team has broad expertise in the clinical, regulatory and commercialization aspects of rare diseases as well as process development and manufacturing for cellular therapies. Members of our management team have held senior positions at Shire, Genzyme, Novartis, Lonza and other companies pursuing development, manufacturing and commercialization of gene and cellular therapies and therapies to treat rare diseases.

Our Strategy

Our goal is to develop and commercialize potentially curative lentiviral-based gene therapies for patients and expand the use of this approach to treat a number of diseases. Key elements of our strategy to achieve our goal include:

- *Rapidly Advance Our Initial Gene Therapies.* We are developing a deep pipeline of four gene therapies to treat Fabry disease, Gaucher disease, Pompe disease and cystinosis. We intend to rapidly advance these gene therapies into clinical trials and obtain initial efficacy data in patients from these development programs. AVR-RD-01 has been well tolerated and demonstrated clinically significant increased enzyme activity to date in an ongoing Phase 1 clinical trial. We received approval from both the Australian and Canadian regulatory authorities to initiate our company-sponsored Phase 2 clinical trial for AVR-RD-01 and, on June 7, 2018, the first patient in this clinical trial was dosed at an Australian site. In addition, we expect enrollment in the United States and Japan to begin in 2019 following receipt of regulatory clearance. Phase 1/2 clinical trials of both AVR-RD-02 and AVR-RD-04 are being planned and we expect patients will be dosed in 2019. In addition, we intend to pursue pathways for accelerated review and approval of our product candidates by the FDA and international regulatory authorities through programs such as the Regenerative Medicine Advanced Therapies, or RMAT, program in the United States.
- *Develop First-Line Gene Therapies for Lysosomal Storage Diseases.* We are initially targeting lysosomal storage diseases and intend to conduct clinical trials in both treatment-experienced and treatment-naïve patient groups in order to maximize the potential of our lentiviral-based gene therapies for patients. We are pioneering the use of a milder conditioning regimen, designed to be performed in an outpatient setting, as we believe this will enable us to pursue early intervention for the treatment of lysosomal storage diseases and expand into a wide range of diseases where lentiviral-based gene therapy has not been previously utilized. We will continue to leverage advancements in stem cell transplantation in order to improve patient tolerability of our lentiviral-based gene therapies.
- *Globally Develop, Manufacture and Commercialize Our Gene Therapies.* Lysosomal storage diseases afflict patients globally and we intend to build global infrastructure in order to provide treatment to patients around the world. We currently intend to conduct clinical trials across multiple geographies, including the United States, Canada, Australia, Japan, Europe and Israel. We have established a global network of suppliers and contract manufacturing organizations, or CMOs.
- *Industrialize Lentiviral-Based Gene Therapy.* We are developing a manufacturing process that is both reproducible and scalable. We believe our innovations in viral vector design, cellular manufacturing and other related processes are important steps towards advancing the field of lentiviral-based gene therapy and realizing its full potential to treat a number of diseases. We intend to leverage our core competencies to implement a closed, automated manufacturing system that will enable us to deliver our gene therapies to patients at an industrialized scale.
- *Leverage Our Approach Beyond Our Initial Indications.* We are initially developing gene therapies for the treatment of four different lysosomal storage diseases and believe that we will gain significant learnings and technical insights from these programs. We intend to leverage our technology and insights to treat a number of rare and non-rare diseases where we believe our lentiviral approach has transformative potential.

Our Approach

We develop therapies utilizing our *ex vivo* lentiviral-based gene therapy approach to transform a patient's own cells into a drug product. Our gene therapies employ lentiviral vectors that are designed to result in stable integration of the desired genes in the chromosomes of the stem cells such that they are permanently maintained in the cell and can be reproduced as the cell divides. We focus on delivering our lentiviral-based gene therapies to hematopoietic stem cells, which are primitive stem cells that develop into all types of blood cells, including white blood cells, red blood cells and platelets. To accomplish this, we extract a patient's hematopoietic stem cells and modify them *ex vivo* to add a new, functional copy of the gene that is defective in the target disease. We then infuse the modified cells back into the patient. Our gene therapies are designed to be administered to the patient as a one-time therapy in an outpatient setting following a milder outpatient conditioning regimen.

We are initially focused on employing our approach to treat and potentially cure lysosomal storage diseases. These diseases have well understood biologies, identified patient populations and represent large markets with approximately $4.0 billion in worldwide net sales in 2017. We are industrializing our *ex vivo* lentiviral-based gene therapy approach into a robust, scalable and, if approved, commercially viable process that will allow us to deliver our potentially curative therapies to patients with these and other serious monogenic disorders.

Advantages of Our Lentiviral-Based Gene Therapy Approach

We believe lentiviral-based gene therapy provides numerous advantages, including:

- *Durable Benefit.* Lentiviral vectors have the potential to provide life-long benefits with a single dose. Lentiviral vectors can integrate stably into the genes of hematopoietic stem cells and, when these cells replicate, they pass the integrated genes on to their progeny cells.
- *Systemic Therapeutic Effect.* Progeny cells circulate systemically and therefore have the ability to provide therapeutic benefit to affected tissues and organs throughout the body.
- *History of Safety.* Over the past 10 years, no instances of insertional mutagenesis or leukemogenesis from lentiviral vectors have been observed in clinical trials in which over 200 total patients have been treated.
- *Broad Patient Applicability.* Lentiviral-based gene therapies have been used to deliver treatments to patients of all ages, including children, and to patients who may be ineligible for other types of gene therapy due to the presence of preexisting antibodies that fight against viral vectors.
- *Larger and Varied Payloads.* In contrast to other viral vectors, lentiviruses have the capacity to carry larger gene sequences, which allow them to potentially address a large variety of indications.

Strategic Selection of Our Initial Indications

There are approximately 50 identified lysosomal storage diseases, which are characterized by an abnormal toxic build-up of by-products in the body's cells. We are initially targeting Fabry disease, Gaucher disease, Pompe disease and cystinsois. Each of these diseases affects a meaningful number of patients, has a suboptimal standard of care and, we believe, is appropriate for lentiviral-based gene therapy. We believe our approach addresses the shortcomings of existing therapies where patients' disease continues to progress despite chronic dosing and that our approach has the potential to cure these diseases.

Clinical proof of concept already exists for allogeneic bone marrow transplant in some lysosomal storage diseases, supporting the notion that transplantation of cells that produce normal enzyme can have clinical impact on disease. Experience with allogeneic bone marrow transplant in patients with Gaucher disease provides evidence to support our *ex vivo* gene therapy approach. Additionally, in cystinosis, transplant of human bone marrow and hematopoietic stem cells into a mouse model demonstrates proof of concept efficacy for transplant. Our *ex vivo* gene therapy approach allows patients to be their own cell donor, eliminating the need to find a matched bone marrow donor, while reducing the risk of complications related to more intensive conditioning regimens and short-term immunosuppressants utilized in allogeneic cell transplant.

Expanding the Utility of Lentiviral-Based Gene Therapy with Outpatient Conditioning

A core part of our approach is to expand the use of lentiviral-based gene therapy to treat numerous diseases. We believe that we will be able to demonstrate durable effects in our targeted diseases with a milder conditioning regimen which has the potential for reduced short- and long-term toxicities. This, in turn, will make lentiviral-based gene therapy a therapeutic option for less acutely severe diseases or diseases with approved therapies in which large unmet medical needs remain.

Prior to the reintroduction of *ex vivo* modified stem cells, a conditioning regimen is generally required to remove cells from the bone marrow. These conditioning regimens create sufficient space in the bone marrow for the modified hematopoietic stem cells to engraft and produce their progeny cells. Ablation requires the use of cytotoxic drugs that can compromise the patient's immune system. The degree of immune system compromise increases with the degree of cell removal, so the need for ablation has historically required a risk/benefit assessment to balance the risks of immune system compromise with potential therapeutic benefit in the targeted disease.

Lentiviral-based gene therapy has been observed to be well-tolerated in third parties' ongoing clinical trials for diseases such as beta thalassemia, ALD and ADA-SCID. Lentiviral vectors also serve as the tool for gene transfer for CAR-T therapies in cancer. Within gene therapy, the use of *ex vivo* lentiviral-based therapies has been restricted primarily to the most acutely severe diseases where risks of the typical requirement for heavily ablating the patients' bone marrow and thus significantly impairing these patients' immune systems had an acceptable risk/benefit profile. The ablation procedure, also known as the conditioning regimen, is administered prior to the gene therapy. The more intensive the conditioning regimen, the greater the risk of toxicity and thus the need for more intensive in-patient monitoring and potential for lengthy hospitalization.

In contrast to other diseases with pathophysiologies where gene therapy requires aggressive conditioning regimens, we believe we can generally achieve sufficient cell engraftment in the lysosomal storage diseases on which we are focused by utilizing a milder conditioning regimen. We believe this approach will lead to less immunosuppression and therefore potentially necessitate only an outpatient conditioning regimen. This outpatient regimen has the potential to improve patient tolerability and extend the reach of *ex vivo* lentiviral-based gene therapy into a number of diseases.

Enhancing Our Gene Therapies and Industrializing Our Manufacturing Capabilities

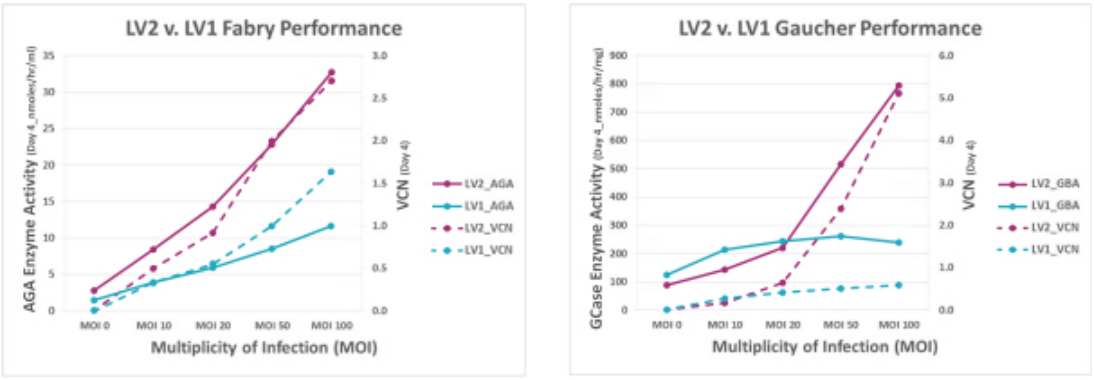
Key to our strategy is to continuously improve our technology and production processes and to leverage these improvements across our gene therapies.

Next Generation Vector Technology

We utilize our core expertise in the development and optimization of lentiviral vectors to continuously improve the vectors used in our gene therapies. We have made and expect to continue to make enhancements to our lentiviral vectors to improve efficacy, efficiency and safety. Our goal is to employ vectors that are state of the art and that can be produced in a cost-effective and scalable manner.

As one example from early, small-scale *in vitro* studies, the figures below show the improved efficacy of our optimized proprietary four-plasmid-produced lentiviral vector, or LV2, over the three-plasmid-produced lentiviral vector, or LV1, for Fabry disease and Gaucher disease, as measured by increases in both vector copy number, or VCN, and enzyme activity. VCN refers to the average number of copies of the lentiviral-vector inserted gene that are integrated into the genome of a cell, and multiplicity of infection refers to a measure of the number of infectious viral particles added to an *in-vitro* cell culture in relation to the total number of cells in the culture. As shown in the figures below, increasing the number of infectious viral particles in a cell culture in order to transduce a constant number of cells results in increases in VCN and enzyme activity levels.

Increased Enzyme Activity and VCN *in vitro* with LV2



Development of Industrialized Manufacturing Processes

We are establishing global manufacturing relationships that will provide us with drug product manufacturing capabilities to support all aspects of the development and eventual commercialization of our gene therapies. We have key manufacturing partnerships in place for the production of plasmids and vectors used in our gene therapies.

Automated, Closed Manufacturing System

Our team has significant experience in cell processing and commercial-scale cellular therapy manufacturing. We have developed and are implementing a detailed plan for the more cost efficient and scalable manufacturing of our gene therapies. In contrast to a number of other gene therapy companies that have not developed their commercial scale plans from the outset, we are currently executing on our plans to move to a closed suspension bioreactor system for vector production as well as a closed, automated system for manufacturing our gene therapy product. We currently have a CMO partner for the production of our cellular drug product in Australia and we have established two CMO partners in the United States who are currently preparing for production with CGMP-readiness in process for AVR-RD-01 and AVR-RD-02. We also plan to establish a CMO partner in Europe.

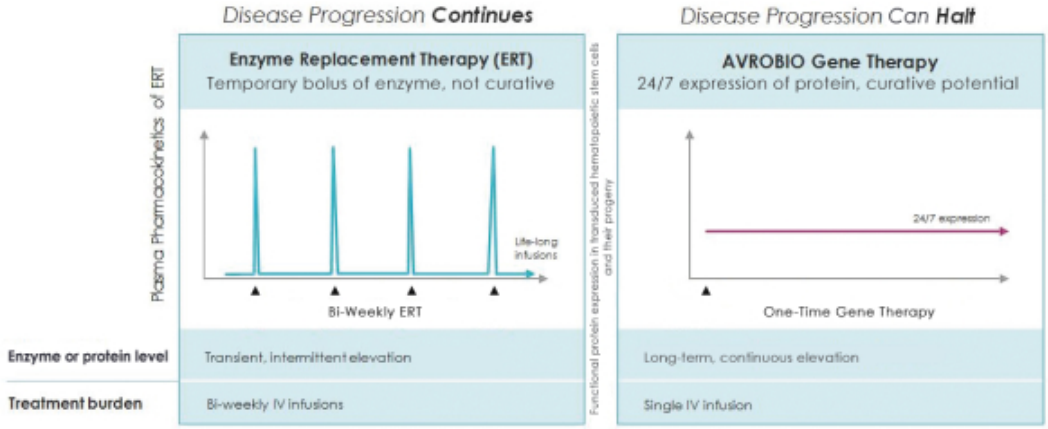
Advantages of Cryopreservation

Our drug product is cryopreserved. In production of our gene therapy for the ongoing Phase 1 clinical trial for AVR-RD-01, greater than 75% cell viability was observed after thaw. Cryopreserved drug product allows for multiple benefits for patients and across the supply chain, including extensive safety testing of the drug product prior to patient administration, convenient patient/clinic scheduling and overall flexibility of supply chain logistics.

Advantages of Our Approach over Existing Therapies

We believe our gene therapy solutions offer several potential advantages over existing therapies for lysosomal storage diseases, including:

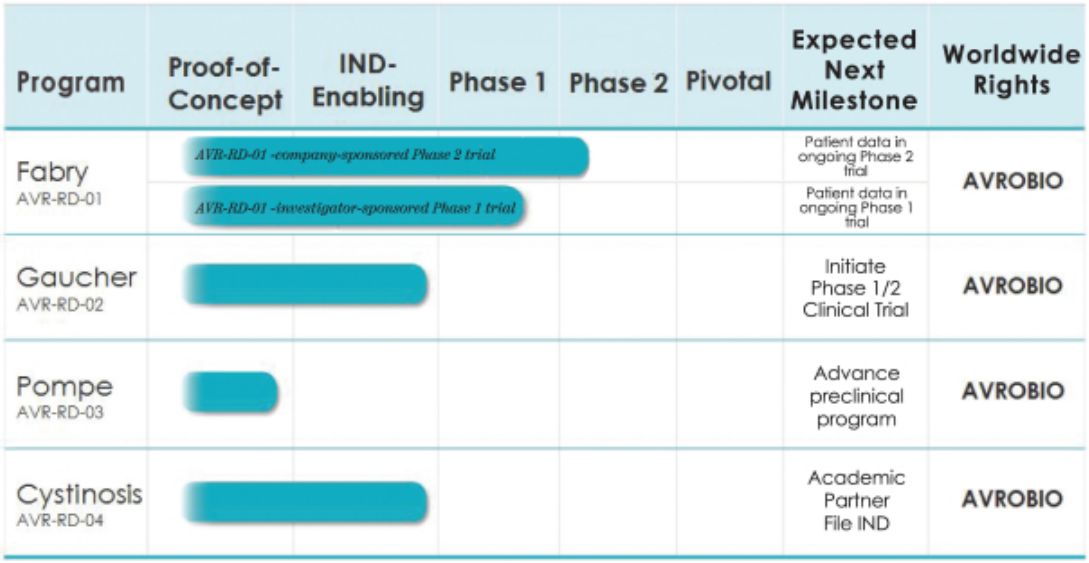
- *Curative Impact that can Halt or Reverse Disease Progression.* Existing ERTs for Fabry, Gaucher and Pompe and oral therapies for cystinosis provide some therapeutic benefit to patients. However, because of their suboptimal pharmacokinetics, these ERTs only temporarily increase plasma enzyme levels and the therapies for cystinosis require multiple doses throughout the day. In contrast, our lentiviral-based gene therapies are designed to cause the body to constantly produce the functional enzyme or other protein. This can potentially halt pathological damage and, depending on the targeted indication and organ system, may even reverse disease progression. Our lentiviral-based gene therapies may provide potentially curative treatment to patients. This concept is illustrated in the graphs below.



- *Durable, Single-Dose Treatment.* Our gene therapies offer the potential for a single dose to replace life-long, bi-weekly infusions or daily oral therapies that are often accompanied by numerous side effects and impact patients' quality of life. Our gene therapies are designed to transform the patient's own cells into a drug product that enables the continuous delivery of functional enzyme or other protein throughout the body after a single dose.
- *Reduced Treatment Cost Over a Patient's Lifetime.* Existing ERTs and oral therapies can cost millions of dollars over a patient's lifetime because these therapies require frequent doses of expensive treatments to manage symptoms. Our single-dose gene therapies are designed to replace the costly chronic intravenous and oral therapies that are the current standard of care for patients with lysosomal storage diseases.

Our Pipeline

Our initial gene therapies are AVR-RD-01 for the treatment of Fabry disease, AVR-RD-02 for the treatment of Gaucher disease, AVR-RD-03 for the treatment of Pompe disease and AVR-RD-04 for the treatment of cystinosis. AVR-RD-01 is currently being evaluated in an investigator-sponsored Phase 1 clinical trial and a company-sponsored Phase 2 clinical trial, which we initiated on June 7, 2018. In addition, Phase 1/2 clinical trials for both AVR-RD-02 and AVR-RD-04 are being planned and we expect patients will be dosed in 2019. The status of our initial lentiviral-based gene therapy programs is reflected below.



AVR-RD-01, Our Gene Therapy for Fabry Disease

We are developing AVR-RD-01 for the treatment of Fabry disease. We manufacture AVR-RD-01 from stem cells that are first extracted from the patient, modified to add the gene that encodes for AGA, and then infused into the patient. AVR-RD-01 is currently being investigated in an investigator-sponsored Phase 1 clinical trial, in which it has been well tolerated and demonstrated increased enzyme activity to-date. On June 7, 2018, we dosed the first patient in our company-sponsored Phase 2 clinical trial.

Disease Overview

Fabry disease is a rare lysosomal storage disease associated with significant morbidity and early mortality. It is caused by a gene defect that causes a deficiency of AGA, which breaks down a particular type of fat in the body's cells known as globotriaosylceramide, or Gb3. As Gb3 and other related substrates increase in patients with Fabry disease, Gb3 becomes toxic to the patient's cells. Gb3 and other glycosphingolipids accumulate and result in damage to the kidneys, heart and brain. Accumulation of Gb3 in tissues such as the heart and the vascular system can lead to life threatening vascular blockages and thus stroke and heart attacks. In addition, high levels of Gb3 substrate accumulation in the kidney can cause kidney failure. Gb3 can also accumulate in other tissues, such as the nervous system where it leads to debilitating pain. Due to end-stage renal disease and other life-threatening complications associated with Fabry disease, the average life expectancy in affected males is approximately 58 years of age.

Most patients with Fabry disease begin experiencing chronic pain in childhood but are often not diagnosed with Fabry disease until their twenties, due to a broad variation in patient symptoms. Over 1,000 gene mutations

associated with Fabry disease have been identified. It is estimated that Fabry disease is diagnosed in approximately one in 40,000 males and one in 118,000 females in the United States, but studies have suggested that a larger number of patients may be undiagnosed.

Fabry disease is an X-linked disorder, with the responsible gene located on the X chromosome. Because males have only one X chromosome, an abnormal copy of the gene that causes Fabry disease is sufficient to cause the disease. However, unlike other X-linked disorders, where female carriers of an abnormal gene are usually unaffected, Fabry disease also often causes significant morbidity in females who inherit one abnormal copy and one normal copy of the gene associated with the disease.

Limitations of Current Therapies

Fabry disease is primarily treated with periodic infusions of ERT consisting of AGA enzyme over the patient's lifetime. The most commonly prescribed ERTs for Fabry disease are Fabrazyme, marketed by Sanofi Genzyme, and Replagal, marketed by Shire. In 2017, Fabrazyme and Replagal generated worldwide net sales of over $880 million and $470 million, respectively. The annual average cost to the healthcare system per patient prescribed Fabrazyme in the United States is approximately $320,000. In addition, because ERTs are not curative and only slow, but do not halt, the progression of disease, patients deteriorate and the healthcare system incurs significant costs associated with recurring medical interventions.

Although ERT provides therapeutic benefit and can reduce Gb3 substrate levels and extend a patient's life expectancy, ERT requires chronic infusions throughout the patient's life. Patients prescribed ERT generally receive an infusion every other week. However, because of their suboptimal pharmacokinetics, ERTs only temporarily increase plasma enzyme levels. As a result, patients with Fabry disease prescribed ERT continue to have disease progression, including ongoing decline in renal function, potentially including renal failure, cardiovascular disease and ongoing debilitating pain including periods of severe pain crisis. Physicians report that patients have recurrence of symptoms as the therapeutic effect of ERT wanes between bi-weekly treatments.

Alternatives to ERT for patients with Fabry disease are limited. Galafold (migalastat), an oral therapy marketed by Amicus, was approved by the European Medicines Agency, or EMA, in May 2016, and Amicus has also submitted a new drug application, or NDA, for migalastat to the FDA. Amicus reports that only 35% to 50% of the gene mutations associated with Fabry disease are amenable to migalastat.

Our Solution

We are developing AVR-RD-01 to halt disease progression and potentially cure patients with Fabry disease with a single dose of the patient's own hematopoietic stem cells modified in an *ex vivo* procedure. AVR-RD-01 is a lentiviral-based gene therapy that contains a codon-optimized human gene and is designed to maximize the likelihood of sustained AGA production by hematopoietic stem cells and their progeny.

We believe that AVR-RD-01 offers a promising treatment for Fabry disease for the following reasons:

- *One-Time Delivery.* Lentiviral-based gene therapy provides the potential to transform a patient's own cells into a drug product that enables the continuous delivery of active enzyme throughout the body after a single dose.
- *Proven Biology.* Years of observations of patients prescribed ERT indicate that even partial plasma AGA activity is associated with improved outcomes. Increased AGA enzyme is able to reduce Gb3 levels in multiple cells and tissues supporting the ability of AGA in the plasma to enter lysosomes and degrade Gb3 in a process referred to as cross correction.
- *Wide Therapeutic Window.* We believe that even partial enzyme activity, if continuous, has the potential to provide long-term therapeutic benefit. A wide range of levels of plasma AGA activity has

been demonstrated to be both safe and effective in preclinical studies, reducing the need for precise regulation of enzyme expression levels and reinforcing that overexpression of AGA is not associated with increased safety risks.

- *Mutation Independent.* AVR-RD-01 is designed to increase plasma AGA levels in a patient's cells, regardless of which of the more than 1,000 specific mutations underlie the patient's disease.

Ongoing Multicenter Clinical Trial

In an ongoing Phase 1 clinical trial of AVR-RD-01 being conducted by the University Health Network, or UHN, at three centers in Canada, up to six patients with Fabry disease who have been treated with ERT for at least six months are expected to be enrolled. In this clinical trial, ERT for these patients is suspended one month prior to receiving AVR-RD-01 and ERT is then resumed one month after the AVR-RD-01 treatment and continued at bi-weekly intervals.

The primary goal for this clinical trial is to assess the safety and toxicity of AVR-RD-01 as measured by the frequency of clinically notable abnormal vital signs and laboratory values and the frequency of treatment-related adverse events. The safety of our out-patient conditioning regimen is also being assessed in this clinical trial.

A secondary objective for this clinical trial is to obtain preliminary efficacy signals of AVR-RD-01 therapy as assessed by AGA enzyme activity. Plasma AGA enzyme activity derived from administration of ERT decreases rapidly after administration with no residual plasma activity remaining approximately one day after treatment. To evaluate the ability of AVR-RD-01 to increase enzyme activity, we assess the level of AGA activity in a patient immediately prior to the administration of the patient's next dose of ERT, when limited or no plasma AGA activity would be expected.

The first two patients in this clinical trial have been dosed and the treatment was well tolerated. In these patients, both of whom are male, the level of plasma AGA enzyme activity began to rise within days of receiving AVR-RD-01, from nearly undetectable levels before treatment to levels above the range for males with classic Fabry disease, defined as less than 1 nmol/hr/ml.

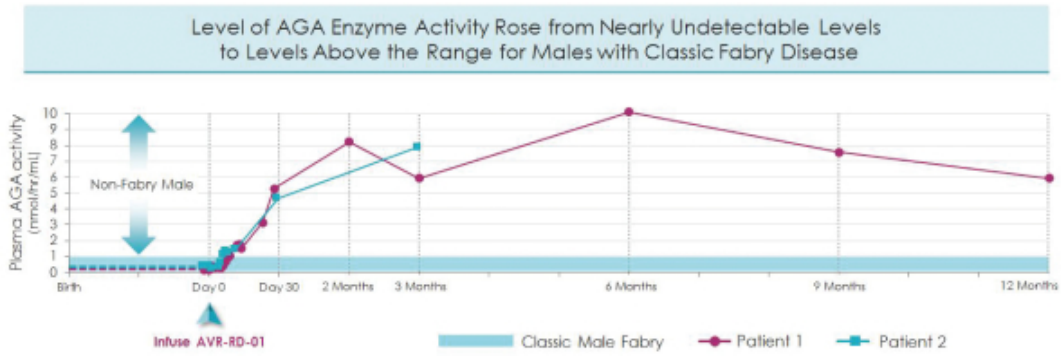
As of twelve months after receiving AVR-RD-01, the first patient's plasma AGA enzyme activity levels continued to be above the range for males with classic Fabry disease. AGA enzyme activity levels in the second patient remained above the range for males with classic Fabry disease as of three months after treatment. We believe these preliminary results support the potential of AVR-RD-01 to drive active enzyme production for long durations.

Plasma AGA Activity (nmol/hr/ml) Following Treatment with AVR-RD-01

	Day 0 (Infusion of AVR-RD-01)	3 Months	12 Months
Patient 1	0.1	5.8	5.8
Patient 2	0.2	7.6	N/A

The below graph illustrates the levels of plasma AGA activity following AVR-RD-01 treatment in the two patients over various points in time.

Expression of Functional Plasma AGA



VCN refers to the average number of copies of the lentiviral-vector inserted gene that are integrated into the genome of a cell. It is typically expressed as an average VCN for a cell population.

We believe the trends in average VCN observed for both AVR-RD-01 and *in vivo* nucleated blood cells in the ongoing Phase 1 clinical trial are consistent with the trends observed in a 2017 published study on hematopoietic stem cell gene therapy for ALD in the *New England Journal of Medicine*. The table below reflects average VCN data observed for the first two patients in the ongoing Phase 1 clinical trial. Importantly, these VCNs have been sufficient to generate plasma AGA enzyme activity in these two patients that was continuously above the range for males with classic Fabry disease.

Average VCN in Patients Dosed with AVR-RD-01 in the Ongoing Phase 1 Clinical Trial

	Patient 1	Patient 2
Drug Product VCN (day 4)	0.68	1.43
1 month VCN	0.42	0.79
3 month VCN	0.55	1.1
6 month VCN	0.35	n/a
9 month VCN	0.26	n/a
12 month VCN	0.16	n/a
14 month VCN	0.12	n/a

Preliminary results from this clinical trial also indicate the presence of lentiviral-vector inserted sequences in the blood and bone marrow of the first patient, which is a signal for successful transduction of the cells. An analysis of the bone marrow from the first patient in this clinical trial measured at 14 months following treatment with AVR-RD-01 suggests engraftment through this period. Because patients in this trial are also receiving ERT, it is not possible to assess the ability of the functional AGA produced by AVR-RD-01 to drive the reduction of substrates, including Gb3 levels, or reduce symptoms of the disease. Altogether, the results observed to date suggest that AVR-RD-01 is capable of delivering and integrating the gene coding for AGA into the human genome and subsequently enabling expression of active AGA enzyme in patients with Fabry disease.

Safety

Preliminary safety data from the first two enrolled patients indicate AVR-RD-01 was generally well-tolerated. As of April 26, 2018, there were 47 adverse events reported, 40 of which were assessed by the investigator as being possibly, probably or definitely related to protocol treatment or procedures (which includes apheresis, stem cell transplant procedure, blood draws, insertion of central catheters, drugs used for mobilization of stem cells and/or conditioning, and the drug product). Only one event, the development of a left thigh mass, was originally reported as a serious adverse event but has now been considered by the study investigator as not serious and has resolved in the patient. In addition, investigators observed a suppression of white blood cell counts and thrombocytopenia in both patients which is an expected outcome based on the conditioning regimen. These decreases were transient and not associated with any negative long-term impact on the patients. Because this clinical trial is ongoing, safety data are preliminary and subject to change. Subsequent to April 26, 2018, we have not been notified by the investigators in this clinical trial of any suspected unexpected serious adverse events. In addition, an independent data monitoring committee has reviewed the one-month post-treatment data of each patient treated with AVR-RD-01 and has approved continuing to enroll patients in this clinical trial.

Phase 2 Multinational Clinical Trial

On June 7, 2018, we dosed the first patient in our open label, multinational Phase 2 clinical trial of AVR-RD-01 in 2018. Enrollment in our Phase 2 clinical trial is ongoing, and we expect to enroll eight to 12 treatment-naïve males, 16 years and older, with classic Fabry disease. Our objectives for this trial are to assess safety and efficacy as measured by multiple indicators, such as Gb3 levels in various tissues, kidney and cardiac function, gastrointestinal symptoms, and pain and quality of life scores. All enrolled patients will receive a single treatment with AVR-RD-01 and will be followed for 48 weeks to measure safety and efficacy. We received approval from both the Australian and Canadian regulatory authorities to initiate our Phase 2 clinical trial for AVR-RD-01. We also plan to submit applications to allow commencement of clinical trials in the United States to the FDA and in Japan to the Pharmaceuticals and Medical Devices Agency, or PMDA, following meetings with each of these respective regulatory authorities. The first patient in our Phase 2 clinical trial was dosed at an Australian site on June 7, 2018.

During a scheduled protocol visit seven days after dosing, the patient reported nausea and vomiting and was admitted to hospital for fluid hydration. Because the nausea and vomiting warranted hospital admission, the event was categorized as a serious adverse event, possibly related to the conditioning regimen; nausea and vomiting have resolved in the patient. A suppression of white blood cell count, which is an anticipated outcome of the conditioning regimen, was also observed in the patient. The treating physician has decided to monitor the patient in hospital until the patient's white blood cell count increases. Because this clinical trial is ongoing, safety data are preliminary and subject to change.

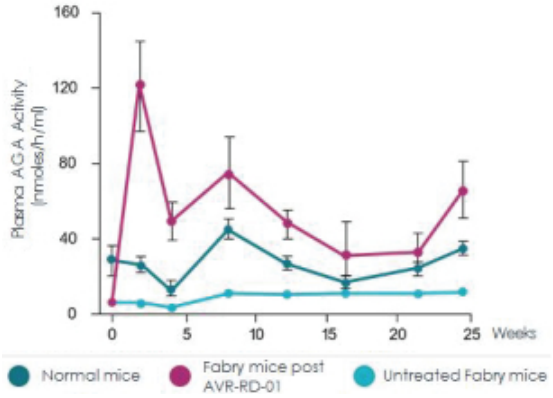
During our Phase 2 clinical trial, we plan to transition the lentiviral vector from LV1 to our optimized proprietary LV2, which we believe will further improve the efficacy and further enhance the safety of our lentiviral-based gene therapy. Because this proposed transition only impacts the *ex vivo* cell transduction process, and not the actual AGA enzyme that is produced by the transduced cells, or drug product, we believe this transition will be supported with *in vitro* comparability studies.

Preclinical Data

AVR-RD-01 has been evaluated in multiple mouse models. Key observations from these preclinical studies serve as the foundation for our lentiviral-based gene therapy approach:

- AGA cross correction occurs by which AGA in plasma was taken up into cells confirming that efficacy of AGA is not limited only to the cells that receive the gene therapy.
- Lentiviral-based gene therapy targeting stem cells in mouse models of Fabry disease led to an elevated and sustained level of AGA enzyme activity in plasma.

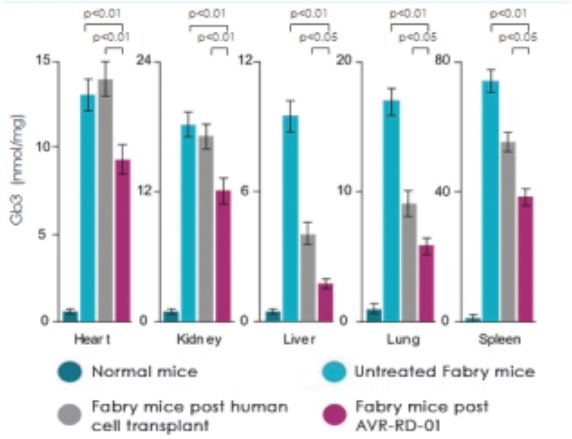
Increased Enzyme Activity in Fabry Mouse Model After Receiving AVR-RD-01



In a direct test of the ability of AVR-RD-01 to correct deficiencies in plasma AGA enzyme activity, a mouse model was created using a mouse strain in which the gene for AGA was inactivated. Human stem cells were extracted from patients with Fabry disease and the gene for AGA was added using a lentiviral vector to create AVR-RD-01. AVR-RD-01 was then introduced to the mice. Twelve weeks following administration, the levels of Gb3 in both the spleen and liver were reduced to a statistically significant extent compared to mice that received unmodified cells from patients with Fabry disease. The FDA utilizes reported statistical measures when evaluating the results of a clinical trial, including statistical significance as measured by p-value as an evidentiary standard of efficacy, to evaluate the reported evidence of a product candidate's safety and efficacy. If not otherwise specified, we used a conventional 5% or lower p-value ($p < 0.05$) to define statistical significance for the data presented in this prospectus. Levels in other tissues such as the heart and kidney also showed downward trends in Gb3. This study demonstrated that:

- Our lentiviral vector could efficiently transform human stem cells into a gene therapy.
- AVR-RD-01 could engraft and replicate to produce progeny cells containing the AGA gene.
- The AGA gene was expressed and functionally active post-treatment.
- Cross correction of AGA produced by cells containing the functional AGA gene caused reductions in Gb3 in various tissues in the mouse model.

Significant Reduction in Gb3 Levels in Multiple Tissues in a NOD/SCID/Fabry Mouse Model After Receiving AVR-RD-01



In addition, in a peer reviewed publication, overexpression of functional AGA with levels as high as 3,000 times the normal range in the mouse model over an 18 month period was not linked to toxicity or adverse effects as determined by long-term animal studies, implying a wide therapeutic window.

AVR-RD-02, Our Gene Therapy for Gaucher Disease

We are developing AVR-RD-02 for the treatment of Type 1 Gaucher disease. We will manufacture AVR-RD-02 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that encodes for glucocerebrosidase, or GCase, and then infused into the patient. We plan to initiate a Phase 1/2 clinical trial for AVR-RD-02 in patients with Type 1 Gaucher disease and expect to dose the first patient in this clinical trial in 2019 following our filing of a Clinical Trial Application, or CTA, in Canada.

Disease Overview

Gaucher disease is a rare, autosomal recessive, lysosomal storage disease caused by a hereditary deficiency of functional GCase, an enzyme responsible for degrading glucocerebroside, a cell membrane building block, into glucose and lipids within lysosomes of cells. In patients with Gaucher disease, the recycling of glucocerebroside from the breakdown of old red and white blood cells is inhibited, leading to its accumulation in macrophages. These abnormal macrophages, known as Gaucher cells, accumulate in multiple organs, particularly the liver, spleen and bone marrow.

Gaucher disease is one of the most common lysosomal storage diseases. It is diagnosed in approximately one in 44,000 births worldwide and is more prevalent in certain ethnic groups, such as people of Ashkenazi Jewish heritage. Approximately 90% of patients suffering from Gaucher disease in western countries have Type 1 Gaucher disease, which manifests as multiple morbidities including enlargement of the spleen and liver, low red blood cells, or anemia, low platelet count, or thrombocytopenia, and bone abnormalities including bone pain, fractures and arthritis. Bruising, risk of bleeding and fatigue are common due to the thrombocytopenia and anemia. Type 1 Gaucher disease does not have manifestations of central nervous system symptoms.

Limitations of Current Therapies

Type 1 Gaucher disease is currently treated with bi-weekly infusions of ERT consisting of recombinant GCase over a patient's lifetime. The most commonly prescribed ERTs for Gaucher disease are Cerezyme, marketed by Sanofi Genzyme, and VPRIV, marketed by Shire.

Although long-term ERT for Gaucher disease results in some therapeutic benefit, ERTs leave patients with significant unmet needs. Twenty-five percent of patients with Gaucher disease continue to experience physical limitations following two years of ERT, and a clinically significant percentage of patients continue to experience bone pain, thrombocytopenia and enlargement of spleen following ten years of ERT. In a published study of ERT therapy for Gaucher disease, six target goals were evaluated, including parameters for hemoglobin and platelet levels, spleen and liver volumes, and general bone pain and severe disabling bone pain known as bone crisis. Following at least four years of ERT in this study, approximately 60% of patients failed to achieve at least one of these target goals. In addition, up to 15% of patients with Gaucher disease develop antibodies that limit the efficacy of the ERT.

In addition to ERTs, the FDA has approved several oral therapies for the treatment of Gaucher disease, including Zavesca (miglustat) marketed by Actelion and Cerdelga (eliglustat) marketed by Sanofi Genzyme. We believe these oral therapies also provide suboptimal treatment. Zavesca is approved as a second line therapy and is associated with significant toxicities, including diarrhea, weight loss and tremors. Cerdelga is not approved for use in children, has highly variable metabolism due to patient-to-patient genetic variations and is highly susceptible to interactions with other drugs.

Both ERTs and oral therapies for Gaucher impose significant costs on the healthcare system. In the United States, the annual average cost to the healthcare system per patient prescribed Cerezyme or VPRIV is between approximately $325,000 and $400,000. The annual average cost to the healthcare system per patient prescribed Cerdelga is approximately $250,000. In 2017, Genzyme's Cerezyme and Cerdelga together generated worldwide net sales of over $1.0 billion and Shire's VPRIV generated worldwide net sales of approximately $350 million.

Our Solution

We are developing AVR-RD-02 to potentially cure patients with Gaucher disease with a single dose of the patient's own hematopoietic stem cells modified in an *ex vivo* procedure. AVR-RD-02 is a lentiviral-based gene therapy that contains a codon-optimized human gene and is designed to maximize the likelihood of sustained GCase production in hematopoietic stem cells and their progeny.

Upcoming Clinical Trial

We plan to initiate a Phase 1/2 clinical trial of AVR-RD-02 in patients with Type 1 Gaucher disease and to begin dosing patients in this clinical trial in 2019. We plan to file a CTA in Canada for this trial in the second half of 2018. Our initial clinical trial will be an adaptive trial that will include both treatment-naïve patients and patients that are currently stable on ERT. We intend to enroll 8 to 16 patients, between the ages of 16 and 35, with Type 1 Gaucher disease. Patients currently prescribed ERT will cease treatment throughout the clinical trial. All enrolled patients will receive a single treatment with AVR-RD-02 and will be followed for 52 weeks to measure safety and efficacy. Our efficacy endpoints for this clinical trial will include measures of clinical efficacy, such as liver and spleen volumes, hemoglobin, platelet counts, bone pain and bone density measures along with other blood markers used in Gaucher disease.

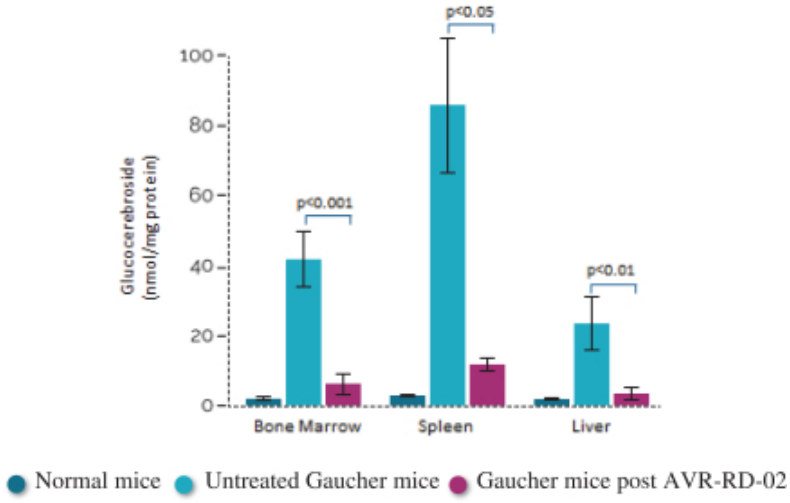
Preclinical Data

AVR-RD-02 is based on extensive preclinical work from our collaborators at Lund University and leverages findings published in 2015 in *Molecular Therapy* which concluded that, in a Gaucher disease mouse model, a lentiviral-based gene therapy containing the gene for GCase could prevent the development and reverse clinically relevant signs of the disease.

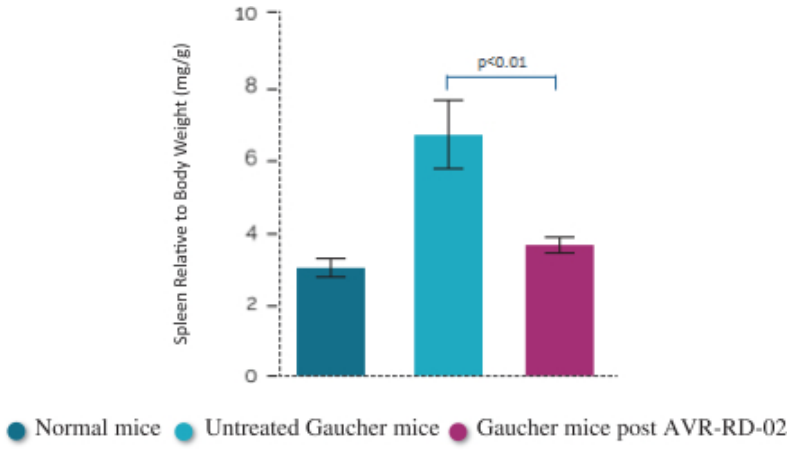
In preclinical studies, a mouse model of Gaucher disease exhibited increased glucocerebroside levels in the clinically-relevant tissues, the bone marrow, spleen and liver, and mimicked many of the same symptoms seen in patients such as an enlarged spleen. These preclinical studies assessed glucocerebroside levels, Gaucher cell

infiltration, and spleen volume in the mouse model over 20 weeks following treatment with AVR-RD-02. When mice with established disease were treated with AVR-RD-02, glucocerebroside levels decreased and symptoms such as an enlarged spleen were reversed within 20 weeks. Over this period, increased enzyme levels were observed in the bone marrow, spleen and liver in the mouse model. In addition, the mice that were treated with AVR-RD-02 prior to manifesting symptoms did not develop symptoms of the disease. These data support the potential efficacy of AVR-RD-02 to prevent, as well as reverse, symptoms in patients with Gaucher disease.

AVR-RD-02 Leads to a Significant Reduction in Glucocerebroside Levels Across Multiple Clinically-relevant Tissues



***Ex Vivo* Lentiviral-based Gene Therapy Leads to a Significant Reduction in Spleen Volume in a Mouse Model of Gaucher Disease**



AVR-RD-03, Our Gene Therapy for Pompe Disease

We are developing AVR-RD-03 for the treatment of Pompe disease. We will manufacture AVR-RD-03 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that encodes for acid alpha glucosidase A, or GAA, attached to a peptide sequence known as a glycosylation-independent lysosomal targeting, or GILT, tag and then infused into the patient. AVR-RD-03 will incorporate a GILT tag because the GILT tag has been found to increase the uptake of GAA into cells, especially in muscle cells by a multiple of 25, which is a particularly important target tissue for patients with Pompe disease.

Disease Overview

Pompe disease is a rare, autosomal recessive lysosomal storage disease caused by a mutation in the gene that encodes for GAA that results in the buildup of glycogen, a complex sugar, in the body's cells. The accumulation of glycogen in certain organs and tissues, especially muscles, impairs normal tissue and organ function. Patients with Pompe disease experience serious muscle related problems, including progressive muscle weakness, especially in the legs and trunk, and the muscles that control breathing. As the disorder progresses, breathing problems can lead to respiratory failure.

The overall diagnosed incidence of Pompe disease is estimated to be approximately one in 58,000 people although frequency and disease progression varies with age of onset, ethnicity and geography. Overall diagnosed incidence of Pompe disease is projected to increase to one in 22,000 people as it is increasingly included in newborn screening panels.

The severity of Pompe disease symptoms and rate of progression is highly variable and correlated with age of symptom onset and the degree of enzyme deficiency. Infantile or early onset disease, the most severe form of Pompe disease, accounts for approximately 25% of all affected patients. Those with early-onset disease are usually diagnosed in the first few months of life. Left untreated, these patients can die due to heart failure, respiratory distress or malnutrition resulting from feeding difficulties within the first year of life. Patients with late-onset disease typically have higher enzyme levels and usually have symptoms such as reduced mobility and respiratory problems. Late-onset patients experience progressive difficulty walking and respiratory decline. While life expectancy can vary, Pompe disease is a life-limiting disease that can result in death due to complications from respiratory failure.

Limitations of Current Therapies

Pompe disease is currently treated with ERT delivered by bi-weekly intravenous infusion. The only approved therapy for Pompe disease is Lumizyme (known as Myozyme outside of the United States), marketed by Sanofi Genzyme, which generated worldwide net sales of over $950 million in 2017. The annual average cost to the healthcare system per patient prescribed Lumizyme in the United States is approximately $500,000.

Though patients treated with ERT for Pompe disease have improved survival and respiratory function, ERT is not curative, and patients in long-term observational studies continue to have increased risk of heart failure and have residual muscle weakness including difficulties swallowing with risk of aspiration. One challenge with ERT treatment for Pompe disease is that a standard dose requires approximately twenty-fold more enzyme compared to standard doses for Fabry or Gaucher diseases. Large doses of Lumizyme that are delivered systemically in order to achieve potentially therapeutic levels in the target tissues result in approximately 90% of patients developing antibodies against the therapy. These antibody responses may impact both the efficacy and safety of Lumizyme. The FDA approval of Lumizyme carries a black box warning related to the risk of severe allergic and immune mediated reactions, including life-threatening anaphylaxis.

Our Solution

We are in early preclinical development of AVR-RD-03 to potentially cure patients with late-onset Pompe disease. We are developing AVR-RD-03 to be a gene therapy product containing a codon-optimized human gene

for GAA attached to a GILT tag designed to increase uptake of GAA in muscle cells. AVR-RD-03 will target patients with late onset Pompe disease, which represent the majority of patients with this disease.

Preclinical Data

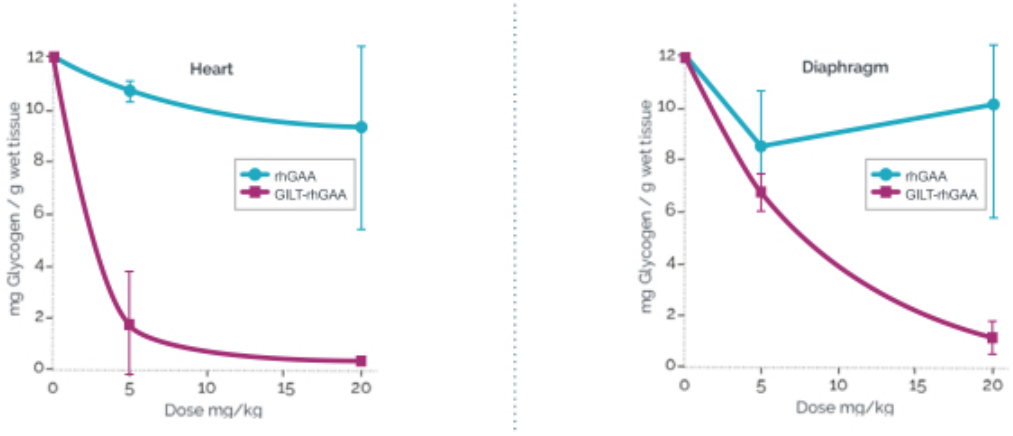
Published preclinical results from a mouse model of Pompe disease support the potential of lentiviral-based gene expression of GAA to prevent some of the symptoms of GAA deficiency. These results also demonstrated the need to further increase the uptake of GAA into muscle cells to treat patients, which is a known challenge for ERTs and leads to the use of large quantities of enzyme to attempt to deliver effective treatment levels.

In these published preclinical results from a mouse model of Pompe disease, treatment utilizing a lentiviral vector encoding GAA led to increased levels of active enzyme across multiple organs and tissues, including clinically relevant tissues such as heart and diaphragm. This enzyme activity was correlated with reductions in glycogen storage in these tissues. While reduction in left ventricular mass and normalization of heart rate were observed in the mouse models seven to eight months following treatment with this lentiviral-based gene expression of GAA, only lesser improvements in other muscles, such as skeletal muscle strength, were observed.

We believe we can use a GILT tag to address the known challenges of skeletal muscle uptake in patients with Pompe disease. Attachment of a GILT tag to a particular protein can increase the effective uptake of the protein into target tissues. We are designing AVR-RD-03 to use a GILT tag to facilitate GAA uptake into cells and thereby reduce the therapeutically required amount of GAA produced by a patient's cells following gene therapy treatment.

In mouse models of Pompe, administration of recombinant GAA with the GILT tag demonstrated significant reduction in glycogen in cardiac and skeletal muscles as compared to the administration of recombinant GAA alone. We licensed GILT tag technology from BioMarin and are incorporating a GILT tag into our lentiviral vector with the goal of the patient producing GILT-tagged GAA following treatment with AVR-RD-03.

GILT-tag Version of Recombinant Human (rh)GAA Impact on Levels of Stored Glycogen Compared to non GILT-tagged Recombinant Human (rh)GAA



AVR-RD-04, Our Gene Therapy for Cystinosis

We are developing AVR-RD-04 for the treatment of patients with cystinosis. We will manufacture AVR-RD-04 from hematopoietic stem cells that are first extracted from the patient, modified to add the gene that

encodes for cystinosin, and then infused into the patient. In a planned academic sponsored Phase 1/2 clinical trial, we expect the first patient will be dosed in 2019.

Disease Overview

Cystinosis is a rare, genetic, autosomal recessive, lysosomal storage disease caused by the accumulation of the amino acid cystine that is produced in the lysosomes of cells as the result of protein degradation. Cystine is normally transported through the lysosomal membrane to the cytosol where it is reutilized after its transformation to cysteine. In cystinosis, cystine accumulates inside the lysosomes because of a defect in the gene that encodes cystinosin, a protein that transports cystine across the lysosomal membrane. Cystine is poorly soluble and forms crystals as its concentration increases. These crystals build up and cause complications in many organs and tissues. The kidneys and eyes are especially vulnerable to damage, and the muscles, thyroid, pancreas and testes may also be affected.

The most severe form of cystinosis begins in infancy, causing poor growth and a particular type of kidney damage in which certain molecules, such as glucose, amino acids, phosphate, and bicarbonate, that should be reabsorbed into the bloodstream are instead eliminated in the urine. These renal problems ultimately lead to impaired growth and may result in soft, bowed bones, especially in the legs. By the time the patient is approximately two years old, cystine crystals may be present in the cornea, and the buildup of these crystals in the eye causes pain and an increased sensitivity to light. Untreated children with cystinosis may experience complete kidney failure by the age of ten. Other signs and symptoms that may occur in untreated patients, especially after adolescence, include muscle deterioration, blindness, inability to swallow, diabetes, thyroid and nervous system problems. More than 90% of untreated patients require a kidney transplant before the age of 20. It is estimated that cystinosis disease is diagnosed in approximately one in 170,000 people.

Limitations of Current Therapies

Cystinosis is currently treated with two oral formulations of cysteamine that enter the lysosome and stimulate the breakdown of cystine into products that do not require the cystinosin protein to be transported. Oral treatment can delay the development of kidney failure by six to ten years if it is started at a very early age, however it cannot prevent kidney failure or the development of other complications, such as the formation of cystine crystals in the cornea. The approved oral therapies for cystinosis are Procysbi (delayed release cysteamine bitartrate), marketed by Horizon Orphan and Cystagon (cysteamine bitartrate) marketed by Mylan. The annual average cost to the healthcare system per patient prescribed Procysbi in the United States is between approximately $625,000 and $750,000.

Procysbi and Cystagon must be taken orally every 12 or six hours, respectively, leading to significant pill burden and compliance challenges. Because cysteamine works by directly binding to cystine, rather than through a typical small molecule that inhibits an enzyme or receptor, a substantial quantity is required. For adults, this can mean taking at least 12 capsules twice a day, every day. Oral therapy with cysteamine is associated with a high degree of noncompliance due to the frequency with which it must be dosed and the accompanying nausea, as well as the acrid sulfur smell that it produces in the breath and body. It has been estimated that only one third of patients are able to adhere to the strict dosing schedule. Studies have shown that adherence diminishes over time in adolescents and adults despite disease impact. Further, oral cysteamine treatment has no effect on ocular cystine crystals deposits, thus requiring patients to be treated with topical cysteamine eye drops which must be applied each hour the patient is awake.

Our Solution

We are developing AVR-RD-04 to potentially cure patients with cystinosis with a single dose of the patient’s own hematopoietic stem cells modified in an *ex vivo* procedure. AVR-RD-04 is a lentiviral-based gene therapy containing a human gene for cystinosin designed to maximize the likelihood of sustained cystinosin production in hematopoietic stem cells and their progeny.

Upcoming Clinical Trial

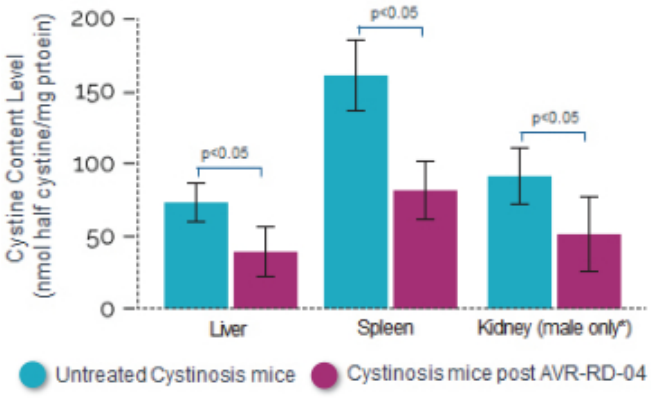
In a planned Phase 1/2 clinical trial of AVR-RD-04 that will be conducted by our collaborators at the University of California, San Diego, six patients with cystinosis who are currently being treated with cysteamine will be enrolled. Cystine levels in tissues such as white blood cells and skin will be followed in these patients as well as cystine crystal counts in the eye. Clinical parameters such as kidney function, muscle strength, bone density, and endocrine function will also be followed with the intent of identifying appropriate parameters to inform future clinical development. We expect that patients enrolled in this trial will undergo a more extensive conditioning regimen instead of the milder conditioning regimen we use for our other target indications. Our collaborators plan to submit an investigational new drug application, or IND, in the U.S. prior to commencing this planned clinical trial.

Preclinical Data

In order to mimic the human disease, the mouse model for cystinosis has the gene for cystinosin disrupted, resulting in the inability of the cystinosin protein to transport cystine out of the lysosomes. This ultimately results in the accumulation of cystine in all tissues similar to that seen in patients with cystinosis. The ability of mouse stem cells modified with a lentiviral vector containing the gene for human cystinosin to treat cystinosis was then tested in the affected mice.

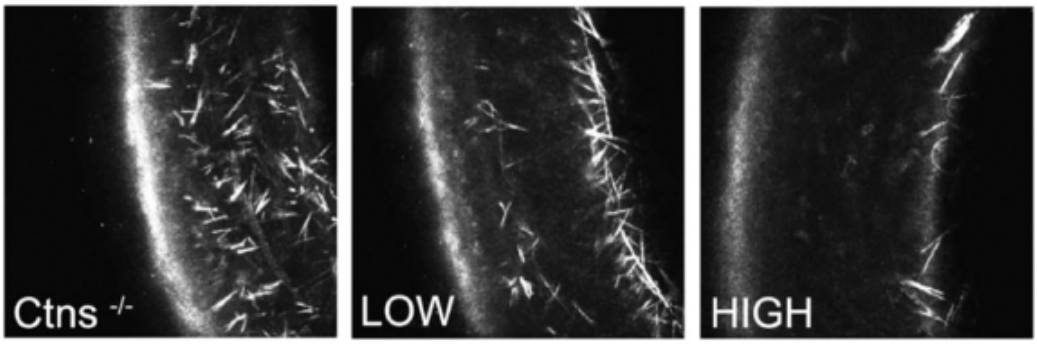
Introduction of AVR-RD-04 into the diseased mice was observed to significantly lower cystine levels in tissues such as the liver, spleen and, in male mice, the kidney. These lower levels persisted until the end of the experiment at eight months. AVR-RD-04 treatment was also associated with potentially improved kidney function in male mice, thus addressing an essential clinical need in patients with cystinosis.

AVR-RD-04 Leads to Lower Cystine Levels in Multiple Tissues in a Mouse Model of Cystinosis



In a separate experiment in this cystinosis mouse model, lentiviral-marked hematopoietic stem cell transplant using normal mouse stem cells led to the reduction of cystine crystals in the cornea, a clinically significant tissue in patients with cystinsosis. In this cystinosis mouse model, affected mice develop eye disease similar to humans, including crystal deposits in the cornea, central corneal opacification, loss of corneal cellular architecture and eventually a scarred, shrunken eye with no function. Published results from this study demonstrate that abundant hematopoetic stem cell-derived macrophages migrated into the cornea and provided functional cystinosin-bearing lysosomes to the corneal cells. The images below reflect the elimination of cystine crystals in the cornea of the mice following engraftment of the modified allogeneic stem cells. As the level of allogeneic stem cell engraftment increased, greater elimination of the cystine crystals was observed. This indicates that stem cells can migrate to the cornea and cross-correct corneal cells. This result demonstrates that it is possible to correct for the defective cystinosin gene in the eye indirectly through expression of the gene in hematopoietic cells.

Transplantation of Allogeneic Hematopoietic Stem Cells Results in Reduction of Cystine Crystals in Corneas of Cystinosis Mice



Manufacturing

Industrializing Our Gene Therapies Through Our Outsourced Manufacture and Supply Network

Our team has leveraged their broad expertise in the manufacturing of gene and cellular therapies to build a global network of CMO partners for the development and manufacture of drug products and outsourced suppliers for the supply of vectors and plasmids. We believe that our third-party CMO partners and suppliers have capacity to accommodate current and future clinical trials and we are continuing to build a global network that will have capacity to generate sufficient quantities to meet our expected commercial needs.

To optimize production of our gene therapies, we are moving our cell processing to an automated, closed system using all disposable supplies. We believe this industrialized manufacturing process will enable a repeatable approach through which we can design and manufacture commercially viable lentiviral gene therapies to potentially treat a large variety of genetic disorders. We expect that our automation of the manufacturing processes will further increase our CMO partners' manufacturing capacity.

Producing a Patient's Gene Therapy

We start the process to produce a patient's gene therapy with the mobilization of a patient's stem cells from the bone marrow to the blood stream and isolate them using a standard procedure used in stem cell transplants. We then treat these cells with a lentiviral vector to insert a functional copy of the gene that is defective in the target disease in a 48-hour process. We preserve patients' modified cells at a very low temperature, using cryopreservation to maintain the cellular material in optimal condition until it is thawed prior to being infused into the patient. The cryopreservation allows us to conduct a number of tests to validate the modified cells prior to introducing them into the patient.

Prior to infusion of the gene therapy-modified cells into the patient, the patients undergo a conditioning regimen to remove some of the patient's unmodified cells from the bone marrow to create sufficient space for the modified hematopoietic stem cells to engraft and produce their progeny. The conditioning regimen used in our approach for AVR-RD-01, AVR-RD-02 and AVR-RD-03 is planned to be completed in an outpatient setting.

After the conditioning regimen is complete, the genetically-modified stem cells are infused into the patient by intravenous administration in an outpatient setting. After infusion, these cells engraft into the bone marrow, replicate and differentiate into various types of blood cells that will distribute throughout the body. These widely distributed cells lead to sustained expression of the desired therapeutic enzyme or other protein. The sustained expression of the functional enzyme or protein is a direct substitute for the protein currently delivered by ERTs, which require periodic infusions.

Intellectual Property and Other Barriers to Entry

The proprietary nature of, or protection for, our gene therapy technology, our product candidates, our production methods and supply chain are an important part of our strategy to develop and commercialize novel therapies. To maximize the commercial opportunity for our gene therapies, if approved, we and our partners have been building and continue to build barriers to entry by our competitors, including:

- We in-license and develop know-how, including data, relating to certain of our product candidates.
- We rely on trade secret protection to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.
- Our management team has significant experience in cell processing and commercial-scale cellular therapy manufacturing. Leveraging this experience, we are building our global network of suppliers and CMO partners which combines their expertise in vector manufacturing, a closed, automated manufacturing system, production of current good manufacturing practices, or CGMP, materials and cryopreservation.
- Our gene therapies are designed to potentially provide a curative benefit. If our gene therapies are approved before any other potentially curative treatments, we believe the benefits of our approach and the resulting first mover advantage may provide meaningful disincentive for companies seeking to develop potentially curative therapies that may compete with our own. See "—Competition."
- We are developing therapies to treat rare diseases and expect to pursue orphan drug designation in the United States and similar protection outside of the United States. These and other regulatory exclusivities, if granted or applicable, can prevent competitors, during the exclusivity period, from obtaining regulatory approval of the same drug or biological product for the same indication. See "—Government Regulation."
- We currently in-license, and we expect to file our own, patents and patent applications relating to certain of our product candidates.

We have in-licensed patents and patent applications from BioMarin Pharmaceutical Inc., and GenStem Therapeutics, Inc., directed to compositions and methods related to the manufacture and use of certain of our gene therapies. In addition, we have in-licensed certain intellectual property rights and know-how from the University Health Network and affiliates of Lund University. For example, we have in-licensed know-how and data related to AVR-RD-01, including certain information about the vector and its use, from University Health Network, and we have in-licensed know-how and data related to AVR-RD-02, including certain information about the vector and its use, from certain professors affiliated with Lund University. Each of our licenses are limited to particular fields, such as Fabry disease, Gaucher disease, Pompe disease, or cystinosis, and are subject to certain retained rights. We do not control the prosecution and maintenance of all of our in-licensed patents and patent applications, and our rights to enforce the patents are limited in certain ways. For additional detail regarding the risks associated with our license agreements see "Risk Factors—Risks Related to Intellectual Property."

As of March 31, 2018, our in-licensed patent portfolio relating to certain of our gene therapies included the following:

- *AVR-RD-03*: two United States (U.S.) patents, projected to expire in 2022 and 2023, and one U.S. patent application, which if granted, would be projected to expire in 2029, as well as corresponding patents and patent applications in certain foreign jurisdictions, as they pertain to compositions and methods for promoting lysosomal uptake of acid alpha-glucosidase and the treatment of Pompe disease. These patents and patent applications are licensed to us by BioMarin and relate to the GILT tag; and
- *AVR-RD-04*: one international patent (PCT) application, which, if pursued and granted in the United States, would be projected to expire in 2038, containing claims directed to hematopoietic stem cells

expressing cystinosin and methods of using the same for the treatment of cystinosis. This patent application is licensed to us by GenStem Therapeutics, and GenStem obtained its rights from the University of California, San Diego.

The term of any given patent depends upon the legal term of patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the application, subject to the timely payment of maintenance fees, among other considerations. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed commonly owned patent. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of FDA regulatory review period. However, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. In certain foreign jurisdictions similar extensions as compensation for regulatory delays are also available. The actual protection afforded by a patent varies on a claim by claim and country by country basis for each applicable product and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent. Currently, we do not own or license patents or patent applications related to our AVR-RD-01 or AVR-RD-02 product candidates. We rely, in some circumstances, on trade secrets and unpatented know-how that is either owned by or licensed to us to protect our technology. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors.

License Agreements

Exclusive License Agreement with University Health Network

In November 2016, we entered into a license agreement with University Health Network, or UHN, pursuant to which UHN granted us an exclusive worldwide license under certain intellectual property rights and a non-exclusive worldwide license under certain know-how, including certain rights to data, in each case subject to certain retained rights, to develop, commercialize and sell products for use in the treatment of Fabry disease. Intellectual property licensed to us under this agreement relates to our Fabry program. In addition, for three years following the execution of the agreement, UHN granted us an exclusive option to obtain an exclusive license under certain improvements to the licensed intellectual property rights as well as an exclusive option to negotiate a license under certain other improvements. Under the terms of the agreement, we are required to meet certain performance milestones within specified timeframes. UHN may terminate the agreement if we fail to meet these performance milestones despite using commercially reasonable efforts and we are unable to reach agreement with UHN on revised timeframes.

As consideration for the licenses, we paid to UHN a one-time upfront fee in the amount of C$75,000 and are obligated to pay an additional annual fee until the first sale of a licensed product in certain markets. We are also required to make payments to UHN in connection with the achievement of certain development and regulatory milestones, in an aggregate amount of C$2.45 million, as well as royalties on a country-by-country basis of a low to mid-single digit percentages on annual sales of licensed products and a lower single digit royalty in certain circumstances. Additionally, we will pay a low double digit percentage of all sublicensing revenue. Our royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the latest to occur of the expiration or termination of the last valid claim under the licensed patent rights in such country (if and when any such patent rights come into existence under the license agreement in the future), the tenth anniversary of the first commercial sale of such licensed product in such country and the expiration of any applicable regulatory exclusivity in such country.

In addition, under this agreement we made a philanthropic commitment to donate funds to organizations for the benefit of the Canadian Fabry community in an amount equal to a low double digit percentage of our royalty payments and regulatory milestone payments, up to a maximum amount of C$500,000 in any calendar year.

Unless terminated earlier, this exclusive license agreement with UHN will expire upon the expiration of our royalty obligation for all licensed products. Either we or UHN may terminate the license agreement if the other party commits a material breach and fails to cure such breach within a certain period of time. UHN may terminate this agreement if we enter into bankruptcy or insolvency. We may terminate this agreement for any reason upon notice to UHN.

License Agreement with Lund University Rights Holders

In January 2017, we entered into an exclusive license agreement with Dr. Stefan Karlsson and Maria Dahl, affiliates of Lund University, pursuant to which Drs. Karlsson and Dahl, and certain other relevant rights holders that may have an interest in intellectual property generated under a research project we are funding with Lund University, granted to us an exclusive worldwide license, subject to certain retained rights, under certain intellectual property rights to develop, commercialize and sell products in any and all uses relevant to Gaucher disease. Intellectual property licensed to us under this agreement relates to our Gaucher program.

As consideration for the license, we are required to make payments in connection with the achievement of certain milestones up to an aggregate of $550,000.

Our license agreement with the rights holders expires on the latest of (i) the twentieth anniversary of the end of a certain research project we are funding pursuant to an agreement with Lund University, (ii) the expiration of the term of any patent filed on the licensed rights that covers a licensed product, (iii) the expiration of any applicable marketing exclusivity right and (iv) such time that neither we nor any of our sublicensees or partners or contractors are commercializing a licensed product. Either we or the rights holders acting together may terminate the license agreement if the other such party commits a material breach and fails to cure such breach within a certain period of time, or if the other party enters into liquidation, becomes insolvent, or enters into composition or statutory reorganization proceedings.

License Agreement with BioMarin Pharmaceutical Inc.

In August 2017, we entered into a license agreement with BioMarin Pharmaceutical Inc., or BioMarin, pursuant to which BioMarin granted us an exclusive worldwide license under certain intellectual property rights related to GILT tags owned or controlled by BioMarin to develop, commercialize and sell Retroviridae-based gene therapy products for use in the treatment of Pompe disease. Under the terms of the agreement, we must use commercially reasonable efforts to develop and commercialize one or more licensed products in the United States and certain European countries. In addition, we are required to initiate an IND-enabling pharmacology/toxicology study of a licensed product within a specified period of time.

As consideration for the license, we paid an initial license fee in the amount of $500,000 and issued 233,765 shares of our Series B preferred stock to BioMarin at the time of our Series B financing. We are also obligated to make payments to BioMarin upon achievement of certain milestones up to an aggregate of $13 million and pay to BioMarin a low single digit royalty percentage on net sales of licensed products covered by patent rights in a relevant country. Our royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the latest to occur of the expiration or termination of the last valid claim under the licensed patent rights in such country, which is currently projected to occur in 2029, the tenth anniversary of the first commercial sale of such licensed product in such country and the expiration of any applicable regulatory exclusivity in such country.

Unless terminated earlier, our license agreement with BioMarin will expire upon the expiration of our royalty obligation for all licensed products throughout the world. Either we or BioMarin may terminate the license agreement if the other party commits a material breach and fails to cure such breach within a certain period of time. BioMarin may also terminate the agreement in the event of any challenge or opposition to the licensed patent rights or related actions brought by us or our affiliates or sublicensees, or if we, our affiliates or sublicensees knowingly assist a third party in challenging or otherwise opposing the licensed patent rights, except as required under a court order or subpoena. In addition, BioMarin may terminate the agreement upon our bankruptcy or insolvency. We may terminate the agreement for any reason upon notice to BioMarin.

License Agreement with GenStem Therapeutics Inc.

In October 2017, we entered into a license agreement with GenStem Therapeutics, Inc., or GenStem, pursuant to which GenStem granted us an exclusive worldwide license, subject to certain retained rights, under certain intellectual property rights owned or controlled by GenStem related to our cystinosis program, including certain rights licensed to GenStem from the University of California, San Diego, to develop, commercialize and sell products for use in the treatment of cystinosis. Under the terms of the agreement, we must use commercially reasonable efforts to develop and commercialize one or more licensed products in the United States and in at least one country from other specified markets. We also agreed to comply with certain access requirements consistent with the California Institute for Regenerative Medicine regulations and to manufacture certain licensed products substantially in the United States.

As consideration for the license, we paid an initial license fee in the amount of $1 million and are required to make payments upon completion of certain development milestones up to an aggregate of $16 million. Additionally, we will pay to GenStem a tiered mid to high-single digit royalty percentage on annual net sales of licensed products as well as a low double-digit percentage of sublicense income received from certain third party sublicensees. Our royalty obligation expires on a licensed product-by-licensed product and country-by-country basis on the eleventh anniversary of the first commercial sale of such licensed product in such country or the expiration of the last valid claim under the licensed patent rights covering such licensed product in such country, which is currently projected to occur in 2038, whichever is later.

Unless terminated earlier, our license agreement with GenStem will terminate upon the expiration of our royalty obligation for all licensed products throughout the world. Either we or GenStem may terminate the license agreement if the other party commits a material breach and fails to cure such breach within a certain period of time. In addition, we may terminate the agreement for any reason upon notice to GenStem.

Competition

Our industry is highly competitive and subject to rapid and significant technological change. Our potential competitors include larger pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as academic institutions, government agencies and private and public research institutions. Key competitive factors affecting the commercial success of our gene therapies are likely to be efficacy, safety and tolerability profile, reliability, convenience, price and reimbursement.

The market for treatment of lysosomal storage diseases is especially large and competitive. The gene therapies we are currently developing, if approved, will face competition.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a small number of our competitors. Accordingly, our competitors may be more successful than we may be in obtaining FDA approval for drugs and achieving widespread market acceptance. Our competitors' products may be more effective, or more effectively marketed and sold, than any product we may commercialize and may render our gene therapies obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our gene therapies. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. Finally, the development of new treatment methods for the diseases we are targeting could render our gene therapies non-competitive or obsolete. See "Risk Factors—Risks related to the discovery and development of our product candidates—We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to successfully market or commercialize any of our product candidates," and elsewhere in this prospectus for more information regarding competitors and competitive products.

Government Regulation

In the United States, biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. Each clinical study protocol for a gene therapy product must be reviewed by the FDA and, in some instances, the National Institute of Health, or NIH, through its Recombinant DNA Advisory Committee, or RAC. FDA approval must be obtained before the marketing of biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER regulates gene therapy products. The CBER works closely with the NIH and its RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in INDs for gene therapies.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

U.S. Biological Products Development Process

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an IND, which must become effective before human clinical studies may begin;
- approval by an independent institutional review board, or IRB, or ethics committee at each clinical study site before each study may be initiated;
- performance of adequate and well-controlled human clinical studies according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with current good manufacturing practices, or CGMPs, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity and, if applicable, the FDA's current good tissue practices, or GTPs, for the use of human cellular and tissue products;
- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the BLA;
- payment of user fees for FDA review of the BLA (unless a fee waiver applies); and
- FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including a gene therapy product, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Where a gene therapy study is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the study is registered with the NIH Office of Science Policy, or OSP, pursuant to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA; however, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the RAC, a federal advisory committee that discusses protocols that raise novel or particularly important scientific, safety or ethical considerations, at one of its quarterly public meetings. The OSP will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OSP web site and may be accessed by the public.

The clinical study sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. An IND is a request for authorization from the FDA to ship an unapproved, investigational product in interstate commerce and to administer it to humans, and must become effective before clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA also may impose clinical holds on a biological product candidate at any time before or during clinical studies due to safety concerns or non-compliance. If the FDA imposes a clinical hold, studies may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Clinical studies involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of

the IND. Clinical studies must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical study must be reviewed and approved by an IRB at or servicing each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical study subject or his or her legal representative and must monitor the clinical study until completed. Clinical research involving recombinant DNA that is subject to NIH guidelines also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Clinical studies typically are conducted in three sequential phases that may overlap or be combined:

- *Phase 1*. The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase 2*. The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- *Phase 3*. Clinical studies are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for approval and product labeling.

Post-approval clinical studies, sometimes referred to as Phase 4 clinical studies, may be conducted after initial marketing approval. These clinical studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire, of study subjects.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. Annual progress reports detailing the results of the clinical studies must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or *in vitro* testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor, acting on its own or based on a recommendation from the sponsor's data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the study period,

the number of patients the FDA will require to be enrolled in the studies in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these studies will be acceptable to the FDA to support marketing approval. The NIH has a publicly accessible database, the Genetic Modification Clinical Research Information System which includes information on gene transfer studies and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these studies.

Concurrent with clinical studies, companies usually complete additional animal studies and also must develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with CGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

After the completion of clinical studies of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. In most cases, the submission of a BLA is subject to a substantial application user fee, although the fee may be waived under certain circumstances. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, for original BLAs, the FDA has ten months from the filing date in which to complete its initial review of a standard application and respond to the applicant, and six months from the filing date for an application with priority review. The FDA does not always meet its PDUFA goal dates, and the review process is often significantly extended by FDA requests for additional information or clarification. This review typically takes twelve months from the date the BLA is submitted to the FDA because the FDA has approximately two months to make a ‘‘filing’’ decision. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with CGMP to assure and preserve the product’s identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary

to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA typically will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with CGMP requirements and adequate to assure consistent production of the product within required specifications. For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with the GTPs. These are FDA regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical studies were conducted in compliance with IND study requirements and GCP requirements. To assure CGMP, GTP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA for a novel product (e.g., new active ingredient, new indication, etc.) must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical studies, sometimes referred to as Phase 4 clinical studies, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product

designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

Expedited Development and Review Programs

The FDA has various programs, including Fast Track designation, breakthrough therapy designation, accelerated approval and priority review, that are intended to expedite or simplify the process for the development and FDA review of drugs and biologics that are intended for the treatment of serious or life-threatening diseases or conditions. These programs do not change the standards for approval but may expedite the development or approval process. To be eligible for fast track designation, new drugs and biological products must be intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product. One benefit of fast track designation, for example, is that the FDA may consider for review sections of the marketing application for a product that has received Fast Track designation on a rolling basis before the complete application is submitted.

Under the breakthrough therapy program, products intended to treat a serious or life-threatening disease or condition may be eligible for the benefits of the Fast Track program when preliminary clinical evidence demonstrates that such product may have substantial improvement on one or more clinically significant endpoints over existing therapies. Additionally, FDA will seek to ensure the sponsor of a breakthrough therapy product receives timely advice and interactive communications to help the sponsor design and conduct a development program as efficiently as possible.

Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review. Under priority review, the FDA's goal is to review an application in six months, compared to ten months for a standard review.

Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be

approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Regenerative Medicine Advanced Therapies Designation

As part of the 21st Century Cures Act, enacted in December 2016, Congress amended the FD&C Act to facilitate an efficient development program for, and expedite review of regenerative medicine advanced therapies, which include cell and gene therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Regenerative medicine advanced therapies do not include those human cells, tissues, and cellular and tissue based products regulated solely under section 361 of the Public Health Service Act and 21 CFR Part 1271. This program is intended to facilitate efficient development and expedite review of regenerative medicine therapies, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and qualify for RMAT designation. A drug sponsor may request that FDA designate a drug as a RMAT concurrently with or at any time after submission of an IND. FDA has 60 calendar days to determine whether the drug meets the criteria, including whether there is preliminary clinical evidence indicating that the drug has the potential to address unmet medical needs for a serious or life-threatening disease or condition. A BLA for a regenerative medicine therapy that has received RMAT designation may be eligible for priority review or accelerated approval through use of surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites. Benefits of RMAT designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A regenerative medicine therapy with RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence from clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval. Like FDA's other expedited development programs, RMAT designation does not change the standards for approval but may expedite the development or approval process.

Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to CGMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the CGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products, include reporting of CGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

We also must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical holds, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors or other stakeholders, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with CGMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain CGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. In addition, a patent can only be extended once and only for a single product. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our patents, if and as applicable, to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant BLA.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods, including some regulatory exclusivity periods tied to patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act, or Affordable Care Act or ACA or PPACA, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 which

created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted four and twelve year exclusivity periods from the time of first licensure of the product. FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product, and FDA will not approve an application for a biosimilar or interchangeable product based on the reference biological product until twelve years after the date of first licensure of the reference product. “First licensure” typically means the initial date the particular product at issue was licensed in the United States. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity, or potency. Therefore, one must determine whether a new product includes a modification to the structure of a previously licensed product that results in a change in safety, purity, or potency to assess whether the licensure of the new product is a first licensure that triggers its own period of exclusivity. Whether a subsequent application, if approved, warrants exclusivity as the “first licensure” of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity.

Government Regulation outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, for example, a Clinical Trial Application, or CTA, must be submitted for each clinical trial to each country's national health authority and an independent ethics committee, much like the FDA and an IRB, respectively. Once the CTA is approved in accordance with a country's requirements, the corresponding clinical study may proceed.

The requirements and process governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical studies must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational product under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the European Union, with the exception of, among other things, region-specific document requirements. The European Union also provides opportunities for market exclusivity. For example, in the European Union, upon receiving marketing authorization, innovative medicinal products generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic or biosimilar application. During the additional two-year period of market exclusivity, a generic or biosimilar marketing authorization can be submitted, and the innovator's data may be referenced, but no generic or biosimilar product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be an innovative medicinal product, and products may not qualify for data exclusivity. Products receiving orphan designation in the European Union can receive ten years of market exclusivity, during which time no similar medicinal product for the same indication may be placed on the market. An orphan product can also obtain an additional two years of market exclusivity in the European Union for pediatric studies. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The criteria for designating an "orphan medicinal product" in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan drug designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if:

- The second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior;
- The applicant consents to a second orphan medicinal product application; or
- The applicant cannot supply enough orphan medicinal product.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical studies must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Other Healthcare Laws and Compliance Requirements

In addition to FDA restrictions on the marketing of pharmaceutical products, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our business or financial arrangements and relationships through which we market, sell and distribute the gene therapies for which we obtain approval. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs; a person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act or federal civil money penalties statute;
- federal civil and criminal false claims laws and civil monetary penalties laws, such as the federal False Claims Act, which impose criminal and civil penalties and authorize civil whistleblower or qui tam actions, against individuals or entities for, among other things: knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent; making, using or causing to be made or used, a false statement or record material to a false or fraudulent claim or obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government;
- the anti-inducement law, which prohibits, among other things, the offering or giving of remuneration, which includes, without limitation, any transfer of items or services for free or for less than fair market value (with limited exceptions), to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary’s selection of a particular supplier of items or services reimbursable by a federal or state governmental program;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payer (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information;
- the federal transparency requirements under the Affordable Care Act, including the provision commonly referred to as the Physician Payments Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members;
- federal government price reporting laws, which require us to calculate and report complex pricing metrics in an accurate and timely manner to government programs; and
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payer. Many U.S. states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payers, including private insurers. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties. Finally, there are state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws.

Violations of fraud and abuse laws may be punishable by criminal and/or civil sanctions, including penalties, fines, imprisonment and/or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private

individuals have the ability to bring actions on behalf of the U.S. government under the federal False Claims Act as well as under the false claims laws of several states.

Law enforcement authorities are increasingly focused on enforcing fraud and abuse laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our current and future business arrangements with third parties, and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including our arrangements with physicians and other healthcare providers, some of whom receive stock options as compensation for services provided, may not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our gene therapies outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

If any of the physicians or other healthcare providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs, which may also adversely affect our business.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payers have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products. For example, in March 2010, the Affordable Care Act was enacted, which, among other things, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care plans; imposed mandatory discounts for certain Medicare Part D beneficiaries as a condition for manufacturers' outpatient drugs coverage under Medicare Part D; subjected drug manufacturers to new annual fees based on pharmaceutical companies' share of sales to federal healthcare programs; imposed a new federal excise tax on the sale of certain medical devices; created a new Patient Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established the Center for Medicare Innovation at the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

Since its enactment, there have been a number of significant changes to the Affordable Care Act. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the Affordable Care Act. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the Affordable Care Act for plans sold through such marketplaces. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of

pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.

The Tax Cuts and Jobs Act of 2017, or TCJA, includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plan, the annual fee imposed on certain health insurance providers based on market share, and the medical device exercise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to reduce the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Congress will likely consider other legislation to replace or modify elements of the Affordable Care Act. We continue to evaluate the effect that the Affordable Care Act and its possible repeal, replacement or further modification could have on our business. It is uncertain the extent to which any such changes may impact our business or financial condition.

In addition, the Budget Control Act of 2011 and the Bipartisan Budget Act of 2015 led to aggregate reductions of Medicare payments to providers of up to 2% per fiscal year that will remain in effect through 2027 unless additional Congressional action is taken. Further, on January 2, 2013, the American Taxpayer Relief Act was signed into law, which, among other things, reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. More recently, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products. Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that additional foreign, federal and state healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in limited coverage and reimbursement and reduced demand for our products, once approved, or additional pricing pressures.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any gene therapies for which we obtain regulatory approval. In the United States and markets in other countries, sales of any gene therapies for which we receive regulatory approval for commercial sale will depend, in part, on the availability of coverage and reimbursement from third-party payers. Third-party payers include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payer will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payer will pay for the product. Third-party payers may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. A decision by a

third-party payer not to cover our gene therapies could reduce physician utilization of our products once approved and have a material adverse effect on our sales, results of operations and financial condition. Moreover, a payer's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In addition, coverage and reimbursement for products can differ significantly from payer to payer. One third-party payer's decision to cover a particular medical product or service does not ensure that other payers will also provide coverage for the medical product or service, or will provide coverage at an adequate reimbursement rate.

As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payer separately and will be a time-consuming process.

Third-party payers are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain and maintain coverage and reimbursement for any product, we may need to conduct expensive clinical trials in order to demonstrate the medical necessity and cost-effectiveness of such product, in addition to the costs required to obtain regulatory approvals. If third-party payers do not consider a product to be cost-effective compared to other available therapies, they may not cover the product as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

Outside of the United States, the pricing of pharmaceutical products is subject to governmental control in many countries. For example, in the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products and medical devices will likely continue as countries attempt to manage healthcare expenditures.

Facilities

Our corporate headquarters are located in Cambridge, Massachusetts. Our current leased facility encompasses approximately 4,580 square feet of office and laboratory space initially, with the ability to expand up to 11,218 square feet during 2018. The lease for this facility expires in January 2023.

Employees

As of June 1, 2018, we had 34 full-time employees, 16 of whom have Ph.D. or M.D. degrees. Of these full-time employees, 24 employees are engaged in research and development activities and ten employees are engaged in finance, legal, human resources, facilities and general management. We have no collective bargaining agreements with our employees and we have not experienced any work stoppages. We consider our relations with our employees to be good.

Legal Proceedings

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this prospectus, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

MANAGEMENT

The following table sets forth the name, age and position of each of our executive officers and directors as of June 15, 2018:

NAME	AGE	POSITION
Executive Officers		
Geoff MacKay	51	President, Chief Executive Officer and Director
Katina Dorton	60	Chief Financial Officer
Nerissa Kreher, M.D.	45	Chief Medical Officer
Non-Employee Directors		
Bruce Booth, D.Phil.(2)	43	Chairman of the Board of Directors
Ian Clark(2)	57	Director
Phillip Donenberg(1)	57	Director
Annalisa Jenkins, M.B.B.S., F.R.C.P.(1)(3)	52	Director
Christopher Paige, Ph.D.(3)	65	Director
Scott Requadt(1)(2)	50	Director
Joshua Resnick, M.D.(3)	42	Director

(1) Member of the audit committee
(2) Member of the compensation committee
(3) Member of the nominating and corporate governance committee

Executive Officers

Geoff MacKay is our co-founder and has been our chief executive officer and director since November 2015. From April 2015 to June 2017, Mr. MacKay served as interim chief executive officer of eGenesis, Inc., a biotechnology company, and from December 2003 to December 2014, he served as chief executive officer of Organogensis Inc., a biotechnology company. Prior to that, from February 1993 to December 2003, Mr. MacKay served in various senior leadership positions within the global transplantation & immunology franchise at Novartis Canada, Global (Basel), USA. Mr. MacKay has served on the board of directors of Replicel, Inc., a regenerative medicine company, since 2016 and previously served as chairman of the board of MassBio, chairman of the board of the Alliance of Regenerative Medicine, and on the advisory council to the Health Policy Commission for Massachusetts. Mr. MacKay holds a B.A in psychology and a graduate certificate in marketing management from McGill University. We believe Mr. MacKay is qualified to serve on our board because of his executive experience in our industry.

Katina Dorton has been our chief financial officer since August 2017. Prior to joining our company, she served as chief financial officer of Immatics GmbH, a biotechnology company, from 2015 to 2017. Ms. Dorton also served as the principle owner of Doric LLC, an advisory firm, from 2011 to 2015, where she provided consulting services to public and private companies in the areas of mergers and acquisitions and strategic finance. Prior to that, she served as managing director at Needham & Co., managing director-investment banking at Morgan Stanley and as an attorney in private practice at Sullivan & Cromwell. Ms. Dorton serves on the board of directors of US Ecology, Inc. Ms. Dorton holds a J.D. from the University of Virginia School of Law, an M.B.A. from George Washington University, and a B.A. from Duke University.

Nerissa Kreher, M.D. has been our chief medical officer since October 2016. Prior to joining our company, Dr. Kreher was the global head of clinical and medical affairs at Zafgen, Inc. from March 2015 to July 2016. Prior to that, from April 2013 to March 2015, Dr. Kreher held roles of increasing responsibility at Shire HGT, most recently as global clinical development lead. From February 2012 to April 2013, Dr. Kreher served as the

executive medical director, metabolic disorders at Alexion Pharmaceuticals, and as senior medical director, medical affairs at Enobia Pharma from January 2011 to June 2012. Dr. Kreher also served as medical director at Genzyme from April 2008 to January 2011 and as director, medical affairs at EMD Serono from April 2006 to April 2008. Dr. Kreher received a B.S. from the University of North Carolina, an M.D. from East Carolina University School of Medicine, an M.S. in Clinical Research from Indiana University and an Executive M.B.A. from Northeastern University.

Non-Employee Directors

Bruce Booth, D.Phil. has served as the chairman of our board of directors since February 2016. Dr. Booth joined Atlas Venture in 2005, and currently serves as partner. Previously, from 2004 to 2005, Dr. Booth was a principal at Caxton Health Holdings L.L.C., a healthcare-focused investment firm, where he focused on the firm's venture capital activities. Prior to Caxton, from 1999 to 2004, he was an associate principal at McKinsey & Company, a global strategic management consulting firm, where he advised clients on R&D productivity, corporate strategy and business development issues across the biopharmaceutical sector. Dr. Booth serves on the board of several privately held companies, as well as on the board of miRagen Therapeutics, Inc. (Nasdaq: MGEN), Zafgen, Inc. (Nasdaq: ZFGN) and Unum Therapeutics Inc. (Nasdaq: UMRX). Dr. Booth also serves on UCB Pharma's New Medicines Scientific Advisory Board and participates on several other advisory boards for pharmaceutical companies and academic medical centers. As a British Marshall Scholar, Dr. Booth holds a D.Phil. in molecular immunology from Oxford University's Nuffield Department of Medicine and a B.S. in biochemistry, summa cum laude, from Pennsylvania State University. We believe Dr. Booth's extensive leadership, executive, managerial and business experience with life sciences companies, including experience in the formation, development and business strategy of multiple start-up companies in the life sciences sector qualifies him to serve on our board of directors.

Ian Clark has served as a member of our board of directors since January 2018. Mr. Clark currently serves as an operating partner within Clarus Ventures. Previously, Mr. Clark served as the chief executive officer and head of North American commercial operations at Genentech from 2010 to 2016. He joined Genentech in 2003 as senior vice president and general manager, BioOncology. In August 2005, he became senior vice president, commercial operations of Genentech. In January 2006, Mr. Clark became executive vice president, commercial operations of Genentech and became a member of its executive committee. Mr. Clark was named head of global product strategy and chief marketing officer of Roche in April 2009. Prior to joining Genentech, Mr. Clark served as general manager of Novartis Canada, overseeing all of the company's country operations, and as chief operating officer for Novartis United Kingdom. Mr. Clark worked in executive positions in sales and marketing for Sanofi and Ivax in the United Kingdom, France and Eastern Europe. Mr. Clark began his career at Searle, where he held management positions in both sales and marketing. Mr. Clark also serves on the board of directors of Agios Pharmaceuticals, Inc., (Nasdaq: AGIO), Corvus Pharmaceuticals, Inc., (Nasdaq: CVRS), and Shire plc, (Nasdaq: SHPG). He has served on the board of directors of the Biotechnology Industry Organization (BIO) since 2009 as well as on the boards of TerraVia and the Gladstone Foundation and as a member of the Federal Reserve Bank of San Francisco's economic advisory council. Mr. Clark received a B.S and honorary doctorate in biological sciences from Southampton University in the United Kingdom. We believe Mr. Clark is qualified to serve on our board because of his industry experience in the field in which we operate and his executive experience with companies in our industry.

Phillip B. Donenberg is a member of our board of directors, effective immediately following the effectiveness of the registration statement of which this prospectus is a part. Effective July 16, 2018, Mr. Donenberg has been appointed Chief Financial Officer and Senior Vice President of Depomed, Inc. Until such date, Mr. Donenberg will continue to serve in his current role as the Senior Vice President and Chief Financial Officer of AveXis, Inc., where he was previously Vice President, Corporate Controller from September 2016 to October 2017. He was the chief financial officer of RestorGenex Corporation from May 2014 to January 2016, when RestorGenex merged with Diffusion Pharmaceuticals LLC and served as the merged company's consultant CFO until September 2016, and the chief financial officer of 7wire Ventures LLC from September 2013 to May

2014. Prior to that time, Mr. Donenberg served as the chief financial officer of BioSante Pharmaceuticals, Inc. from July 1998 to June 2013, when BioSante merged with ANIP Pharmaceuticals, Inc. Mr. Donenberg also has experience serving on the boards of directors of privately held companies. Mr. Donenberg holds a B.S. in Accountancy from the University of Illinois Champaign-Urbana College of Business and is a Certified Public Accountant. We believe Mr. Donenberg is qualified to serve on our board of directors because of his financial expertise and his experience as an executive of companies in the industry in which we operate.

Annalisa Jenkins, M.B.B.C., F.R.C.P. has served as a member of our board of directors since March 2018. Dr. Jenkins has served as the chief executive officer of PlaqueTec Ltd. since November 2017 and was previously the chief executive officer and a member of the board of directors of Dimension Therapeutics, Inc., from September 2014 until its sale to Ultragnyx Pharmaceutical in November 2017. From October 2013 to March 2014, Dr. Jenkins served as executive vice president, head of global research and development for Merck Serono Pharmaceuticals, a biopharmaceutical company. Previously, from September 2011 to October 2013, she served as Merck Serono's executive vice president, global development and medical, and was a member of Merck Serono's executive committee. Prior to that, Dr. Jenkins pursued a 15-year career at Bristol-Myers Squibb Company, a biopharmaceutical company, where, from July 2009 to June 2011, she was a senior vice president and head of global medical affairs. Dr. Jenkins is currently a committee member of the science board to the FDA, which advises FDA leadership on complex scientific and technical issues. Dr. Jenkins serves on the board of directors of Ardelyx, Inc. (Nasdaq: ARDX), Silence Therapeutics (Nasdaq: SLN), Oncimmune (Nasdaq: ONC) and a number of privately held biotech and life science companies. Dr. Jenkins graduated with a degree in medicine from St. Bartholomew's Hospital in the University of London and subsequently trained in cardiovascular medicine in the UK National Health Service. Earlier in her career, Dr. Jenkins served as a medical officer in the British Royal Navy. We believe Dr. Jenkins is qualified to serve on our board based on her industry experience in the field in which we operate and her executive experience with companies in our industry.

Christopher Paige, Ph.D. has served as a member of our board of directors since January 2016. Dr. Paige is a professor in the departments of medical biophysics and immunology at the University of Toronto. In 1997, he served as the vice president, research of the University Health Network and now serves as senior scientist. In 1990, Dr. Paige became the founding director of the Arthritis and Autoimmunity Research Centre as well as director of research at The Wellesley Hospital. He became a member of the Basel Institute for Immunology in Switzerland in 1980 where he worked until joining the Ontario Cancer Institute as a senior scientist in 1987. Dr. Paige earned a B.S. in biology at the University of Notre Dame in 1974 and a Ph.D. in immunology at the Sloan-Kettering Division of Cornell University Graduate School of Medical Sciences in 1979. We believe Dr. Paige is qualified to serve on our board because of his scientific and industry experience in the field in which we operate.

Scott Requadt has served as a member of our board of directors since July 2016. Mr. Requadt is currently a managing director at Clarus, a life sciences investment fund. Mr. Requadt has 17 years of operating and investment experience in the pharmaceutical industry. Prior to joining Clarus in 2005, Mr. Requadt was Director, Business Development of TransForm Pharmaceuticals, and previously practiced for several years as a mergers and acquisitions attorney at the New York City-based law firm of Davis Polk & Wardwell. Before that, Mr. Requadt was a law clerk for a senior judge at the Supreme Court of Canada. Mr. Requadt holds a B.Com (Joint Honors, Economics & Finance) from McGill University, an LL.B from University of Toronto and an M.B.A. from Harvard Business School (Baker Scholar). Mr. Requadt has been involved in multiple Clarus investments spanning both therapeutics and medtech, as well as several research and development risk-sharing collaborations with large pharmaceutical partners. He is currently also a director of VBI Vaccines (Nasdaq: VBIV), ESSA Pharmaceuticals (Nasdaq: EPIX) and Edev S.a.r.l. and has previously been active on the board of directors of TyRx, Catabasis (Nasdaq: CATB), Oxford Immunotec (Nasdaq: OXFD), Link Medicine and Biolex Therapeutics. We believe Mr. Requadt is qualified to serve on our board because of his industry experience as a biotech public and private company investor.

Joshua Resnick, M.D. has served as a member of our board of directors since July 2016. Dr. Resnick has been a partner at SV Health Investors, or SV, since January 2016. Before joining SV in January 2016,

Dr. Resnick was president and managing partner at MRL Ventures Fund, or MRL Ventures, an early-stage therapeutics-focused corporate venture fund that he built and managed within Merck & Co from December 2014 to January 2016. Prior to MRL Ventures, Dr. Resnick was a venture partner with Atlas Venture, or Atlas, focusing on company formation, Seed and Series A investing. During his tenure at Atlas, Dr. Resnick was also the founder and chief executive officer of two start-ups in the immuno-oncology and neuro spaces. Prior to Atlas, Dr. Resnick was a partner at Prism Venture Partners, where he focused on early-stage biopharmaceutical, medical device, tools and diagnostics investments. Dr. Resnick is also an attending physician at Massachusetts General Hospital, as well as Brigham and Women's Hospital since 2006, and an instructor in medicine at Harvard Medical School. Dr. Resnick serves on the board of directors of Kalvista Pharmaceuticals, Inc. (Nasdaq: KALV). Dr. Resnick graduated Magna Cum Laude with a B.A. from Williams College and received his M.D. from the University of Pennsylvania School of Medicine and his M.B.A. from The Wharton School of Business. We believe Dr. Resnick is qualified to serve on our board because of his industry experience as a biotech public and private company investor.

Composition of Our Board of Directors

Our board of directors currently consists of eight members, each of whom is a member pursuant to the board composition provisions of our current certificate of incorporation and agreements with our stockholders. These board composition provisions will terminate upon the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors. Our nominating and corporate governance committee and our board of directors may therefore consider a broad range of factors relating to the qualifications and background of nominees. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is the identification of persons who will further the interests of our stockholders through their established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape, professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Director Independence

Our board of directors has determined that all members of the board of directors, except Mr. MacKay, are independent directors, including for purposes of the rules of The Nasdaq Global Market and the Securities and Exchange Commission, or SEC. In making such independence determination, our board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances that our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director. In considering the independence of the directors listed above, our board of directors considered the association of our directors with the holders of more than 5% of our common stock. Upon the completion of this offering, we expect that the composition and functioning of our board of directors and each of our committees will comply with all applicable requirements of The Nasdaq Global Market and the rules and regulations of the SEC. There are no family relationships among any of our directors or executive officers. Mr. MacKay is not an independent director under these rules because he is an executive officer of our company.

Staggered Board

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering, our board of directors will be divided

into three staggered classes of directors and each will be assigned to one of the three classes. At each annual meeting of the stockholders, a class of directors will be elected for a three-year term to succeed the directors of the same class whose terms are then expiring. The terms of the directors will expire upon the election and qualification of successor directors at the annual meeting of stockholders to be held during the years 2019 for Class I directors, 2020 for Class II directors and 2021 for Class III directors.

- Our Class I directors will be Christopher Paige, Scott Requadt and Joshua Resnick;
- Our Class II directors will be Ian Clark and Annalisa Jenkins; and
- Our Class III directors will be Bruce Booth, Phillip Donenberg and Geoff MacKay.

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering will provide that the number of our directors shall be fixed from time to time by a resolution of a majority of our board of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Board Leadership Structure and Board's Role in Risk Oversight

Currently, the positions of chairman of the board and that of Chief Executive Officer are separated. We believe that separating these positions will allow our Chief Executive Officer to focus on our day-to-day business, while allowing our chairman of the board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as our chairman, particularly as the board of directors' oversight responsibilities continue to grow. While our amended and restated by-laws and corporate governance guidelines do not require that our chairman and Chief Executive Officer positions be separate, our board of directors believes that having separate positions will be the appropriate leadership structure for us following the completion of this offering.

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks more fully discussed in the section entitled "Risk Factors" appearing elsewhere in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Committees of Our Board of Directors

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee, each of which will operate pursuant to a charter adopted by our board of

directors and will be effective upon the effectiveness of the registration statement of which this prospectus is a part. Upon the effectiveness of the registration statement of which this prospectus is a part, the composition and functioning of all of our committees will comply with all applicable requirements of the Sarbanes-Oxley Act of 2002, Nasdaq and SEC rules and regulations.

Audit Committee

Phillip Donenberg, Annalisa Jenkins and Scott Requadt serve on the audit committee, which is chaired by Mr. Donenberg. Our board of directors has determined that Dr. Jenkins and Messrs. Donenberg and Requadt are "independent" for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and each has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated Mr. Donenberg as an "audit committee financial expert," as defined under the applicable rules of the SEC. The audit committee's responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving audit and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee's review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

Compensation Committee

Ian Clark, Bruce Booth and Scott Requadt serve on the compensation committee, which is chaired by Mr. Clark. Our board of directors has determined that each member of the compensation committee is "independent" as defined in the applicable Nasdaq rules. The compensation committee's responsibilities include:

- annually reviewing and recommending to the board of directors the corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and based on such evaluation, recommending to the board of directors (i) determining the cash compensation of our Chief Executive Officer and (ii) grants and awards to our Chief Executive Officer under equity-based plans;
- reviewing and approving the cash compensation of our other executive officers;

- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and recommending to the board of directors the compensation of our directors;
- preparing our compensation committee report if and when required by SEC rules;
- reviewing and discussing annually with management our "Compensation Discussion and Analysis," if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention or termination of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

Nominating and Corporate Governance Committee

Annalisa Jenkins, Christopher Paige and Joshua Resnick serve on the nominating and corporate governance committee, which is chaired by Dr. Jenkins. Our board of directors has determined that each member of the nominating and corporate governance committee is "independent" as defined in the applicable Nasdaq rules. The nominating and corporate governance committee's responsibilities include:

- developing and recommending to the board of directors, criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- developing and recommending to the board of directors, a code of business conduct and ethics and a set of corporate governance guidelines; and
- overseeing the evaluation of our board of directors and management.

Our board of directors may from time to time establish other committees.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Corporate Governance

We have adopted, effective upon the effectiveness of the registration statement of which this prospectus is a part, a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on the investor relations section of our website, which is located at www.avrobio.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

EXECUTIVE COMPENSATION

Executive Compensation Overview

As an emerging growth company, we have opted to comply with the executive compensation disclosure rules applicable to "smaller reporting companies," as such term is defined in the rules promulgated under the Securities Act. This section provides an overview of the compensation awarded to, earned by, or paid to our principal executive officer and our next two most highly compensated executive officers in respect of their service to our company for our fiscal year ended December 31, 2017. We refer to these individuals as our 2017 named executive officers. Our 2017 named executive officers are:

- Geoff MacKay, our President and Chief Executive Officer;
- Katina Dorton, our Chief Financial Officer; and
- Nerissa Kreher, M.D., our Chief Medical Officer.

Our executive compensation program is based on a pay for performance philosophy. Compensation for our executive officers is composed primarily of the following main components: base salary; bonus; and equity incentives in the form of options. Our executive officers, like all full-time employees, are eligible to participate in our health and welfare benefit plans. As we transition from a private company to a publicly traded company, we intend to evaluate our compensation values and philosophy and compensation plans and arrangements as circumstances require. Compensation plans or arrangements that we adopt following the completion of this offering may be materially different from those described in this section.

2017 Summary Compensation Table

The following table sets forth information regarding compensation awarded to, earned by, or paid to our 2017 named executive officers for services rendered to us in all capacities during the fiscal year ended December 31, 2017.

NAME AND PRINCIPAL POSITION	YEAR	SALARY ($)	BONUS ($)(1)	OPTION AWARDS ($)(2)	ALL OTHER COMPENSATION ($)	TOTAL ($)
Geoff MacKay, *President and Chief Executive Officer*	2017	408,000	163,200	30,306	—	601,506
Katina Dorton, *Chief Financial Officer*(3)	2017	113,333	37,917	286,705	24,856(4)	462,811
Nerissa Kreher, M.D., *Chief Medical Officer*	2017	336,600	84,150	18,165	—	438,915

(1) Amounts reflect annual bonuses earned based upon achievement of company and individual performance metrics for the year ended December 31, 2017, but paid in 2018.

(2) Amounts reflect the grant date fair value of option awards granted or modified in 2017 in accordance with the Financial Accounting Standards Board's Accounting Standards Codification Topic 718, or ASC 718. Such grant date fair value does not take into account any estimated forfeitures related to service-vesting conditions. For information regarding assumptions underlying the valuation of equity awards, see Note 2 to our financial statements and the discussion under "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Significant Judgments and Estimates—Stock-based Compensation" included elsewhere in this prospectus. These amounts do not correspond to the actual value that may be recognized by the 2017 named executive officers upon vesting of applicable awards.

(3) Ms. Dorton commenced her employment with us in August 2017. Her annual salary and bonus were prorated to reflect her partial year of service.

(4) Amount reflects our reimbursement of travel and relocation expenses.

Narrative to the 2017 Summary Compensation Table

Base Salary

We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our 2017 named executive officers. Base salaries for our named executive officers are reviewed annually by our compensation committee, typically in connection with our annual performance review process, and adjusted from time to time, based on the recommendation of the compensation committee, to realign salaries with market levels after taking into account individual responsibilities, performance and experience. None of our 2017 named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

Annual Bonus

We currently do not have a formal performance-based bonus plan but intend to adopt our Senior Executive Cash Incentive Bonus Plan in connection with this offering. Our employment agreements with our 2017 named executive officers provide that the executive may be eligible to earn an annual performance bonus of up to a target percentage of the executive's base salary, as described further below under the section entitled "—Employment Arrangements with our Chief Executive Officer and our 2017 Named Executive Officers." From time to time, our board of directors or compensation committee may approve annual bonuses for our named executive officers based on individual performance, company performance or as otherwise determined appropriate.

Equity Compensation

Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them in the form of stock options.

We typically grant stock option awards at the start of employment to each executive and our other employees. We award our stock options on the date our board of directors approves the grant. We set the option exercise price and grant date fair value based on our per-share estimated valuation on the date of grant. For grants in connection with initial employment, vesting begins on the initial date of employment. To date, we have not maintained a practice of granting additional equity on an annual basis, but we have retained discretion to provide additional targeted grants in certain circumstances.

Employment Arrangements with our Chief Executive Officer and our 2017 Named Executive Officers

We have entered into employment agreements with each of our 2017 named executive officers. These agreements set forth the initial terms and conditions of each executive's employment with us, including base salary, target annual bonus opportunity and standard employee benefit plan participation. In connection with this offering, we have entered into an amended and restated employment agreement with each of Mr. MacKay, Dr. Kreher and Ms. Dorton. The amended and restated employment agreements will be effective as of the closing of this offering.

These existing and new employment agreements provide for "at will" employment. The material terms of the new employment agreements with our 2017 named executive officers are described below. The terms "change of control," "cause" and "good reason" referred to below are defined in the applicable employment agreement.

Geoff MacKay

We entered into an employment agreement with Geoff MacKay, our President and Chief Executive Officer, on December 22, 2016. In connection with this offering, we entered into an amended and restated employment agreement with Mr. MacKay, which will become effective as of the closing of this offering. Under the terms of the new employment agreement, Mr. MacKay is entitled to receive an annual base salary of $500,000 and an annual target bonus of 50% of his annual base salary based upon our board of directors' assessment of Mr. MacKay's performance and our attainment of targeted goals as set by the board of directors in its sole discretion. Mr. MacKay also previously entered into a Confidentiality and IP Assignment Agreement with us, the terms of which are incorporated into his new employment agreement.

Mr. MacKay's new employment agreement provides that, in the event that his employment is terminated by us without "cause" or by Mr. MacKay with "good reason", subject to the execution and effectiveness of a separation agreement and release, he will be entitled to receive (i) an amount equal to 100% of his base salary, provided that Mr. MacKay has not breached any of the confidentiality, noncompetition or cooperation provisions set forth in, or incorporated into, the new employment agreement, payable on our normal payroll cycle, and (ii) reimbursement of COBRA premiums for health benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Mr. MacKay had he remained employed with us for up to 12 months. Additionally, all stock options and other stock based awards held by Mr. MacKay that would have vested if he had remained employed by us for an additional 12 months following the date of termination will vest and become exercisable or non-forfeitable as of the date of termination.

Under the new employment agreement, in the event of a "change in control" all stock options and other stock-based awards granted to Mr. MacKay at least 12 months prior to the effective date of the new employment agreement shall accelerate and become fully exercisable or non-forfeitable immediately prior to the change in control. In addition, in the event that Mr. MacKay is terminated by us without "cause" or by Mr. MacKay for "good reason" within three months prior to or 18 months after a change in control, subject to the execution and effectiveness of a separation agreement and release, he will be entitled to receive (i) an amount equal to 150% of the sum of his base salary plus target bonus for that year, and (ii) reimbursement of COBRA premiums for health benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Mr. MacKay had he remained employed with us for up to 18 months. Additionally, all then unvested stock options and other stock-based awards granted to Mr. MacKay will vest and become exercisable or non-forfeitable as of the date of termination.

Nerissa Kreher, M.D.

We entered into an employment agreement with Dr. Nerissa Kreher, our Chief Medical Officer, on November 1, 2016. In connection with this offering, we entered into an amended and restated employment agreement with Dr. Kreher which will become effective as of the closing of this offering. Under the terms of the new employment agreement, Dr. Kreher is entitled to receive an annual base salary of $365,650 and an annual target bonus of 25% of her annual base salary based upon our board of directors' assessment of Dr. Kreher's performance and our attainment of targeted goals as set by the board of directors in its sole discretion. Dr. Kreher also previously entered into a Confidentiality and IP Assignment Agreement with us, the terms of which are incorporated into her new employment agreement.

Dr. Kreher's new employment agreement provides that, in the event that her employment is terminated by us without "cause" or by Dr. Kreher with "good reason", subject to the execution and effectiveness of a separation agreement and release, she will be entitled to receive (i) an amount equal to 75% of her base salary, provided that Dr. Kreher has not breached any of the confidentiality, noncompetition or cooperation provisions set forth in, or incorporated into, the new employment agreement, payable on our normal payroll cycle, and (ii) reimbursement of COBRA premiums for health benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Dr. Kreher had she remained

employed with us for up to nine months. Additionally, all stock options and other stock based awards held by Dr. Kreher that would have vested if she had remained employed by us for an additional nine months following the date of termination will vest and become exercisable or non-forfeitable as of the date of termination.

Under the new employment agreement, in the event of a "change in control" all stock options and other stock-based awards granted to Dr. Kreher at least 12 months prior to the effective date of the new employment agreement shall accelerate and become fully exercisable or non-forfeitable immediately prior to the change in control. In addition, in the event that Dr. Kreher is terminated by us without "cause" or by Dr. Kreher for "good reason" within three months prior to or 18 months after a change in control, subject to the execution and effectiveness of a separation agreement and release, she will be entitled to receive (i) an amount equal to 100% of the sum of her base salary, plus target bonus for that year, and (ii) reimbursement of COBRA premiums for health benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Dr. Kreher had she remained employed with us for up to 12 months. Additionally, all then unvested stock options and other stock-based awards granted to Dr. Kreher will vest and become exercisable or non-forfeitable as of the date of termination.

Katina Dorton

We entered into an employment agreement with Katina Dorton, our Chief Financial Officer, on July 20, 2017. In connection with this offering, we entered into an amended and restated employment agreement with Ms. Dorton which will become effective as of the closing of this offering. Under the terms of the new employment agreement, Ms. Dorton is entitled to receive an annual base salary of $360,000 and an annual target bonus of 40% of her annual base salary based upon our board of directors' assessment of Ms. Dorton's performance and our attainment of targeted goals as set by the board of directors in its sole discretion. Ms. Dorton also previously entered into a Confidentiality and IP Assignment Agreement with us, the terms of which are incorporated into her new employment agreement.

Pursuant to Ms. Dorton's existing and new employment agreements, Ms. Dorton is entitled to reimbursement of temporary living and travel expenses in connection with traveling to and temporary living in Massachusetts of up to $4,000 per month until the earlier of August 28, 2018 and the sale of her Raleigh, North Carolina residence. Ms. Dorton is also entitled to a one-time relocation payment of up to $100,000, less any reimbursements for temporary living and travel expenses previously paid to her, in connection with the relocation of her primary residence from North Carolina to Massachusetts.

Ms. Dorton's new employment agreement provides that, in the event that her employment is terminated by us without "cause" or by Ms. Dorton with "good reason", subject to the execution and effectiveness of a separation agreement and release, she will be entitled to receive (i) an amount equal to 75% of her base salary, provided that Ms. Dorton has not breached any of the confidentiality, noncompetition or cooperation provisions set forth in, or incorporated into, the new employment agreement, payable on our normal payroll cycle, and (ii) reimbursement of COBRA premiums for health benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Ms. Dorton had she remained employed with us for up to nine months. Additionally, all stock options and other stock based awards held by Ms. Dorton that would have vested if she had remained employed by us for an additional nine months following the date of termination will vest and become exercisable or non-forfeitable as of the date of termination.

Under the new employment agreement, in the event of a "change in control" all stock options and other stock-based awards granted to Ms. Dorton at least 12 months prior to the effective date of the new employment agreement shall accelerate and become fully exercisable or non-forfeitable immediately prior to the change in control. In addition, in the event that Ms. Dorton is terminated by us without "cause" or by Ms. Dorton for "good reason" within three months prior to or 18 months after a change in control, subject to the execution and effectiveness of a separation agreement and release, she will be entitled to receive (i) an amount equal to 100% of the sum of her base salary plus target bonus for that year, and (ii) reimbursement of COBRA premiums for health

benefit coverage, up to an amount equal to the monthly employer contribution that we would have made to provide health insurance to Ms. Dorton had she remained employed with us for up to 12 months. Additionally, all then unvested stock options and other stock-based awards granted to Ms. Dorton will vest and become exercisable or non-forfeitable as of the date of termination.

Outstanding Equity Awards at 2017 Fiscal Year-End

The following table sets forth information concerning outstanding equity awards held by our 2017 named executive officers as of December 31, 2017. All equity awards set forth in the table below were granted under our Amended and Restated 2015 Stock Option and Grant Plan, or 2015 Plan.

	Option Awards				Stock Awards	
Name	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date	Number of Shares or Units of Stock that Have Not Vested (#)	Market Value of Shares or Units that Have Not Vested ($) (5)
Geoff MacKay	75,629	166,384(1)	0.41	4/12/2026	72,604(4)	296,950
	—	45,999(2)	0.91	6/12/2027	—	—
Katina Dorton	—	158,318(2)	0.91	7/19/2027	—	—
Nerissa Kreher, M.D.	25,058	60,856(3)	1.20	10/24/2026	—	—
	—	27,571(2)	0.91	6/12/2027	—	—

(1) The shares underlying this stock option vest as follows: 25% of the shares vested on July 1, 2017 and the remainder vest in equal quarterly installments until the option is fully vested on July 1, 2020, subject to the continued employment of the executive officer.

(2) The shares underlying this stock option vest as follows: 25% of the shares vest on the first anniversary of the grant date and the remainder vest in equal monthly installments until the option is fully vested on the fourth anniversary of the grant date, subject to the continued employment of the executive officer.

(3) The shares underlying this stock option vest as follows: 25% of the shares vested on October 3, 2017 and the remainder vest in equal monthly installments until the option is fully vested on October 3, 2020, subject to the continued employment of the executive officer.

(4) On November 27, 2015, Mr. MacKay transferred his ownership of 72,604 shares to each of his two children, 36,302 shares subject to each transfer remain unvested and subject to vesting as of December 31, 2017, based on Mr. MacKay's continued service to our company.

(5) There was no public market for our common stock as of December 31, 2017. This column represents the value of the shares of restricted stock as of December 31, 2017, based on the fair market value of our common stock as of December 31, 2017, which was $4.09 per share.

Compensation Risk Assessment

We believe that although a portion of the compensation provided to our executive officers and other employees is performance-based, our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

Employee Benefit and Equity Compensation Plans

Amended and Restated 2015 Stock Option and Grant Plan

The 2015 Plan, was approved by our board of directors and our stockholders on July 21, 2016. The 2015 Plan was most recently amended in January 2018 with the approval of both our board of directors and our

stockholders. Under the 2015 Plan, we have reserved for issuance an aggregate of 2,008,564 shares of our common stock. The number of shares of common stock reserved for issuance is subject to adjustment in the event of any merger, consolidation, sale of all or substantially all of our assets, reorganization, recapitalization, reclassification, stock dividend, stock split, reverse stock split or other similar transaction.

The shares of common stock underlying awards that are forfeited, canceled, reacquired by us prior to vesting, satisfied without the issuance of stock or otherwise terminated (other than by exercise) and shares of common stock that are withheld upon exercise of an option or settlement of an award to cover the exercise price or tax withholding are added back to the shares of common stock available for issuance under the 2015 Plan.

Our board of directors has acted as administrator of the 2015 Plan. The administrator has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, and to determine the specific terms and conditions of each award, subject to the provisions of the 2015 Plan. Persons eligible to participate in the 2015 Plan are those employees, officers and directors of, and consultants and advisors to, our company as selected from time to time by the administrator in its discretion.

The 2015 Plan permits the granting of (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, or the Code and (2) options that do not so qualify. The per share option exercise price of each option will be determined by the administrator but may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each option will be fixed by the administrator. The administrator will determine at what time or times each option may be exercised. In addition, the 2015 Plan permits the granting of restricted shares of common stock, unrestricted shares of common stock, and restricted stock units.

The 2015 Plan provides that upon the occurrence of a “sale event,” as defined in the 2015 Plan, our board of directors may take one or more of the following actions as to some or all awards outstanding under the 2015 Plan: (i) provide that outstanding options awards will be assumed or substituted by the acquiring or successor corporation, (ii) provide that all unexercised options will terminate immediately prior to the consummation of the sale event unless exercised by the optionee (to the extent exercisable) within a specified period prior to the consummation of the sale event, (iii) make or provide for a cash payment to the optionees equal to the difference between the per share cash consideration in the sale event and the per share exercise price of the outstanding award, (iv) provide that all restricted stock and unvested restricted stock unit awards (other than those becoming vested as a result of the sale event) will be assumed or substituted by the acquiring or successor corporation (v) provide that all restricted stock and unvested restricted stock unit awards (other than those becoming vested as a result of the sale event) will terminate immediately prior to the effective time of any sale event unless repurchased at a price per share equal to the lower of the original per share purchase price paid by the holder (subject to adjustment) or the current fair market value of such shares, determined immediately prior to the effective time of the sale event, (vi) make or provide for a cash payment to the holders of restricted stock or restricted stock unit awards in an amount equal to the consideration payable per share of stock pursuant to the sale event times the number of shares subject to such award. We may also make or provide for a cash payment to participants holding options in an amount equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options (to the extent then exercisable).

The administrator may amend or discontinue the 2015 Plan at any time, subject to stockholder approval where such approval is required by applicable law. The administrator of the 2015 Plan may also amend or cancel any outstanding award, provided that no amendment to an award may adversely affect a participant’s rights without his or her consent.

The 2015 Plan will terminate automatically upon the earlier of 10 years from the date on which the 2015 Plan was adopted by our board of directors or 10 years from the date the 2015 Plan is approved by the Company’s stockholders. As of March 31, 2018, options to purchase 1,788,750 shares of common stock were outstanding under the 2015 Plan. Our board of directors has determined not to make any further awards under the 2015 Plan following the pricing of this offering.

2018 Stock Option and Incentive Plan

Our 2018 Stock Option and Incentive Plan, or the 2018 Plan, was adopted by our board of directors on June 1, 2018 and approved by our stockholders on June 7, 2018 and became effective upon the effectiveness of the registration statement of which this prospectus is part. The 2018 Plan replaces the 2015 Plan as our board of directors has determined not to make additional awards under the 2015 Plan following the pricing of our initial public offering. The 2018 Plan allows the compensation committee to make equity-based and cash-based incentive awards to our officers, employees, directors and other key persons (including consultants).

We have initially reserved 616,300 shares of our common stock, or the Initial Limit, for the issuance of awards under the 2018 Plan. The 2018 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2019, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee, or the Annual Increase. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2018 Plan are authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without any issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2018 Plan and the 2015 Plan will be added back to the shares of common stock available for issuance under the 2018 Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options shall not exceed the Initial Limit cumulatively increased on January 1, 2019 and on each January 1 thereafter by the lesser of the Annual Increase for such year or 1,785,100 shares of common stock.

The 2018 Plan is administered by our compensation committee. Our compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2018 Plan. Persons eligible to participate in the 2018 Plan are those full or part-time officers, employees, non-employee directors and other key persons (including consultants) as selected from time to time by our compensation committee in its discretion.

The 2018 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code, and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed 10 years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right may not be less than 100% of the fair market value of the common stock on the date of grant.

Our compensation committee may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our compensation committee may also grant shares of common stock that are free from any restrictions under the 2018 Plan. Unrestricted stock may be granted to participants in recognition of past services or other valid consideration and may be issued in lieu of cash compensation due to such participant. Our compensation committee may grant cash bonuses under the 2018 Plan to participants, subject to the achievement of certain performance goals.

Our compensation committee may grant awards that vest or become payable upon the attainment of performance goals that are established by our compensation committee and related to one or more performance criteria. The performance criteria that could be used with respect to any such awards include: achievement of specified research and development, publication, clinical and/or regulatory milestones, earnings before interest, taxes, depreciation and amortization, net income (loss) (either before or after interest, taxes, depreciation and/or amortization), changes in the market price of our common stock, economic value added, funds from operations or similar measures, sales or revenue, acquisitions or strategic transactions, operating income (loss), cash flow (including, but not limited to, operating cash flow and free cash flow), return on capital, assets, equity, or investment, stockholder returns, return on sales, gross or net profit levels, productivity, expense, margins, operating efficiency, customer satisfaction, working capital, earnings (loss) per share of stock, sales or market shares and number of customers, any of which may be measured either in absolute terms or as compared to any incremental increase or as compared to results of a peer group.

The 2018 Plan provides that in the case of, and subject to, the consummation of a "sale event" as defined in the 2018 Plan, all outstanding awards may be assumed, substituted or otherwise continued by the successor entity. To the extent that the successor entity does not assume, substitute or otherwise continue such awards, then (i) all stock options and stock appreciation rights will automatically become fully exercisable and the restrictions and conditions on all other awards with time-based conditions will automatically be deemed waived, and awards with conditions and restrictions relating to the attainment of performance goals may become vested and non-forfeitable in connection with a sale event in the compensation committee's discretion and (ii) upon the effectiveness of the sale event, the 2018 Plan and all awards will automatically terminate. In the event of such termination, (i) individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) prior to the sale event; or (ii) we may make or provide for a cash payment to participants holding options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights (to the extent then exercisable).

Our board of directors may amend or discontinue the 2018 Plan and our compensation committee may amend the exercise price of options and amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose but no such action may adversely affect rights under an award without the holder's consent. Certain amendments to the 2018 Plan require the approval of our stockholders. No awards may be granted under the 2018 Plan after the date that is 10 years from the date of stockholder approval. Our board of directors has granted stock options to purchase 75,613 shares of common stock under the 2018 Plan, effective as of the effectiveness of the 2018 Plan.

2018 Employee Stock Purchase Plan

Our 2018 Employee Stock Purchase Plan, or the ESPP, was adopted by our board of directors on June 1, 2018 and approved by our stockholders on June 7, 2018 and became effective upon the effectiveness of the registration statement of which this prospectus is part. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423(b) of the Code. The ESPP initially reserves and authorizes the issuance of up to a total of 223,200 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2019 and each January 1 thereafter through January 1, 2028, by the least of (i) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31; (ii) 1,115,700 shares or (iii) such number of shares as determined by the ESPP administrator. The number of shares reserved under the ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees who have completed at least three months of employment and whose customary employment is for more than 20 hours per week are eligible to participate in the ESPP. However, any employee who owns 5% or more of the total combined voting power or value of all classes of stock is not eligible to purchase shares under the ESPP.

We will make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will usually begin on each January 1 and July 1 and will continue for six-month periods, referred to as offering periods. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the ESPP may purchase shares by authorizing payroll deductions of up to 10% of his or her base compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase shares on the last business day of the offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of common stock, valued at the start of the purchase period, under the ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under the ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The ESPP may be terminated or amended by our board of directors at any time. An amendment that increases the number of shares of common stock authorized under the ESPP and certain other amendments require the approval of our stockholders.

Senior Executive Cash Incentive Bonus Plan

On June 1, 2018, our board of directors adopted the Senior Executive Cash Incentive Bonus Plan, or the Bonus Plan. The Bonus Plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or corporate performance goals, as well as individual performance objectives.

Our compensation committee may select corporate performance goals from among the following: achievement of specified research and development, publication, clinical and/or regulatory milestones, adjusted billings, earnings before interest, taxes, depreciation and amortization, net income (loss) (either before or after interest, taxes, depreciation and/or amortization), changes in the market price of our common stock, economic value-added, funds from operations or similar measure, sales or revenue, acquisitions or strategic transactions, operating income (loss), cash flow (including, but not limited to, operating cash flow and free cash flow), return on capital, assets, equity, or investment, stockholder returns, return on sales, gross or net profit levels, productivity, expense, margins, operating efficiency, customer satisfaction, working capital, earnings (loss) per share of stock, sales or market shares and number of customers, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, or as compared to results of a peer group.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The corporate performance goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the corporate performance goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period. Subject to the rights contained in any agreement between the executive officer and us, an executive officer must be employed by us on the bonus payment date to be eligible to receive a bonus payment. The Bonus Plan also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion and provides the compensation committee with discretion to adjust the size of the award as it deems appropriate to account for unforeseen factors beyond management's control that affected corporate performance.

401(k) Plan

We maintain a tax-qualified retirement plan that provides eligible employees with an opportunity to save for retirement on a tax-advantaged basis. All participants' interests in their contributions are 100% vested when contributed. Contributions are allocated to each participant's individual account and are then invested in selected investment alternatives according to the participants' directions. The retirement plan is intended to qualify under Section 401(a) of the Code. Matching contributions to the plan are made at the discretion of our board of directors.

DIRECTOR COMPENSATION

The following table presents the total compensation for each person who served as a non-employee member of our board of directors and received compensation for such service during the fiscal year ended December 31, 2017. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2017. We reimburse non-employee members of our board of directors for reasonable travel expenses. Mr. MacKay, our President and Chief Executive Officer, did not receive any compensation for his service as a member of our board of directors in 2017. Mr. MacKay's compensation for service as an employee for fiscal year 2017 is presented in "Executive Compensation—2017 Summary Compensation Table."

NAME	FEES EARNED OR PAID IN CASH ($)	OPTION AWARDS ($)	TOTAL ($)
Bruce Booth, D.Phil.	—	—	—
Ian Clark(1)	12,500	176,624	189,124
Christopher Paige, Ph.D.	—	—	—
Joshua Resnick, M.D.	—	—	—
Scott Requadt	—	—	—

(1) Pursuant to a letter agreement with us, Mr. Clark is paid an annual cash retainer of $50,000 for his service on the board of directors. As of December 31, 2017, Mr. Clark held an option to purchase 48,740 shares of our common stock, no portion of which was vested as of such date.

Non-Employee Director Compensation Policy

Our board of directors has adopted a non-employee director compensation policy, effective upon effectiveness of the registration statement of which this prospectus forms a part, that is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering, as set forth below:

	NON-CHAIRMAN MEMBER ANNUAL FEE ($)	CHAIRMAN ANNUAL FEE ($)
Board of Directors	50,000	80,000
Audit Committee	2,500	5,000
Compensation Committee	2,500	5,000
Nominating and Corporate Governance Committee	2,500	5,000

In addition, each non-employee director serving on our board of directors upon the effectiveness of the registration statement of which this prospectus is a part and each non-employee director thereafter first elected or appointed to our board of directors will be granted 18,743 on the date of such effectiveness or of such director's election or appointment to the board of directors, as applicable, which will vest in equal monthly installments over a three year period, subject to the director's continued service through such vesting date(s). On the date of each annual meeting of stockholders of our company, each non-employee director will be granted 9,371, which will vest in full upon the earlier to occur of the first anniversary of the date of grant or the date of our following annual meeting of stockholders, subject to continued service as a director through such vesting date.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under “Executive Compensation” and “Director Compensation” in this prospectus and the transactions described below, since November 17, 2015 (date of inception), there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of five percent or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

License Agreements and Related Agreements with University Health Network

On January 27, 2016, we entered into an exclusive license agreement with University Health Network, or UHN, pursuant to which UHN granted us an exclusive license to certain intellectual property rights relating to Interleukin-12 proteins, or IL-12. We entered into an amendment to this agreement on September 28, 2017. Under this agreement, we paid C$264,000 to UHN upon execution of the agreement which consisted of an upfront license fee and reimbursement of certain patent expenses. We are also obligated to pay an annual license fee as well as payments in connection with the achievement of certain performance and development milestones for an aggregate total of up to C$19.275 million in milestone payments. Additionally, we will pay a low to midsingle digit royalty percentage on annual sales of licensed products, and a low double digit percentage of all sublicensing revenue. For the years ended December 31, 2016 and 2017, we paid $736,000 and $151,000 to UHN under this agreement, respectively. Pursuant to this agreement, UHN also purchased 1,161,665 shares of our common stock for an aggregate purchase price of $480.00 under a stock purchase agreement. Under the terms of the stock purchase agreement, we are obligated to pay to UHN five percent of the proceeds from this offering, up to a cap of $2 million, upon the closing of this offering.

On January 27, 2016, we entered into an option agreement with UHN pursuant to which UHN granted us an exclusive option to enter into an exclusive license under certain intellectual property rights related to Fabry disease. On November 4, 2016, we executed our option and entered into an exclusive license agreement with UHN. Under this agreement, UHN granted us an exclusive worldwide license under certain intellectual property rights and a non-exclusive worldwide license under certain know-how, in each case subject to certain retained rights, to develop, commercialize and sell products for use in the treatment of Fabry disease. Under the terms of the agreement, we paid to UHN a one-time upfront fee of C$75,000 and are obligated to pay an annual maintenance fee until the first sale of a licensed product in certain markets. We are also required to make payments to UHN in connection with the achievement of certain development and regulatory milestones in an aggregate amount of up to C$2.45 million, as well as royalties on a country-by-country basis of a low to midsingle digit percentage on annual sales of licensed products and a lower single digit royalty in certain circumstances. Additionally, we will pay a low double digit percentage of all sublicensing revenue. We also made a philanthropic commitment to donate funds to organizations for the benefit of the Canadian Fabry community in an amount equal to a low double digit percentage of our royalty payments and regulatory milestone payments, up to a maximum of C$500,000 in any calendar year. For the years ended December 31, 2016 and 2017, we paid $87,000 and $16,000 to UHN in connection with this agreement, respectively, which consisted of our license option fee, the upfront fee and maintenance fees. See “Business—License Agreements—Exclusive License Agreement with University Health Network” for further information regarding the 2016 Fabry license agreement with UHN. In connection with this agreement, we also entered into (i) a letter agreement with UHN on November 4, 2016, pursuant to which we agreed to provide certain funding and costs and expenses associated with a clinical trial conducted by UHN for the treatment of Fabry disease, and (ii) a letter agreement with UHN on June 2, 2017, pursuant to which we agreed to provide additional funding and costs and expenses associated with the clinical trial conducted by UHN for the treatment of Fabry disease.

In connection with the above agreements, we have also entered into two separate sponsored research agreements with UHN, one in March 2017 and one in July 2017. The March 2017 agreement was amended and

restated and subsequently amended in November 2017. Pursuant to each of these sponsored research agreements, we agreed to fund certain research projects related to IL-12 and Fabry disease, including salaries of certain researchers of up to C$200,000 and C$164,652 under the March 2017 and July 2017 agreements, respectively.

At the time we entered into each of the above agreements with UHN, UHN was a greater than 5% beneficial owner of our outstanding capital stock. Additionally, Christopher Paige is a senior scientist at UHN and is currently a member of our board of directors. As an inventor of certain of the intellectual property rights related to IL-12 that we license from UHN, Dr. Paige is entitled to a portion of the consideration that we pay to UHN pursuant to the IL-12 license agreement.

Private Placements of Securities

Series Seed Preferred Stock Financing

In January 2016, we sold an aggregate of 3,333,333 shares of our Series Seed preferred stock at a purchase price of $0.45 per share. The following table summarizes purchases of our Series Seed preferred stock by related persons:

STOCKHOLDER	SHARES OF SERIES SEED PREFERRED STOCK	TOTAL PURCHASE PRICE
Atlas Venture Fund X, L.P.(1)	3,333,333	$1,499,999.85

(1) Bruce Booth, D.Phil., a partner at Atlas Venture, is a member of our board of directors. Atlas Venture is a holder of five percent or more of our capital stock.

Series A Preferred Stock Financing

In July 2016, we sold 5,714,286 shares of Series A preferred stock, at a price of $1.3125 per share, pursuant to a stock purchase agreement entered into with the investors. In March 2017, we amended certain provisions of our Series A preferred stock purchase agreement and issued a preferred stock dividend in the form of 3,720,864 additional shares of Series A preferred stock to such investors, which effectively repriced the outstanding shares of Series A preferred stock and changed the purchase price for future shares of Series A preferred stock to be sold under the Series A preferred stock purchase agreement to $0.7949 per share. Concurrent with the amendment, we issued 4,403,070 additional shares of Series A preferred stock at a purchase price of $0.7949 per share. In October 2017, we issued 17,612,279 additional shares of Series A preferred stock in a subsequent closing, at a purchase price of $0.7949 per share.

The following table summarizes purchases of our Series A preferred stock and the issuance of the preferred stock dividend referenced above by related persons:

STOCKHOLDER	SHARES OF SERIES A PREFERRED STOCK	TOTAL PURCHASE PRICE
Atlas Venture Fund X, L.P.(1)	12,580,199	$ 9,999,999.53
Entities affiliated with SV Life Sciences Fund(2)(3)	9,435,150	$ 7,500,000.91
Clarus Life Sciences III, L.P.(4)	9,435,150	$ 7,500,000.91

(1) Bruce Booth, D.Phil., a partner at Atlas Venture, is a member of our board of directors. Atlas Venture is a holder of five percent or more of our capital stock.
(2) Joshua Resnick, M.D., a partner at SV Health Investors, is a member of our board of directors. SV Health Investors is a holder of five percent or more of our capital stock.
(3) Consists of (1) 9,122,809 shares of Series A preferred stock held by SV Life Sciences Fund VI, L.P. and (2) 312,341 shares of Series A preferred stock held by SV Life Sciences Fund VI, Strategic Partners L.P.

(4) Scott G. Requadt, J.D., MBA, a Managing Director at Clarus, is a member of our board of directors. Clarus is a holder of five percent or more of our capital stock.

Series B Preferred Stock Financing

In January 2018, we sold an aggregate of 28,519,322 shares of our Series B preferred stock at a purchase price of $2.1389 per share, pursuant to agreements entered into with the investors. The following table summarizes purchases of our Series B preferred stock by related persons:

STOCKHOLDER	SHARES OF SERIES B PREFERRED STOCK	TOTAL PURCHASE PRICE
Atlas Venture Fund X, L.P.(1)	3,740,239	$ 7,999,997
Entities affiliated with SV Life Sciences Fund(2)(3)	1,870,119	$ 3,999,998
Clarus Life Sciences III, L.P.(4)	2,805,179	$ 5,999,997
Citadel Multi-Strategy Equities Master Fund Ltd.(5)	5,610,360	$11,999,999
Cormorant Private Healthcare Fund I, LP(6)(7)	5,610,360	$11,999,999

(1) Bruce Booth, D.Phil., a partner at Atlas Venture, is a member of our board of directors. Atlas Venture is a holder of five percent or more of our capital stock

(2) Joshua Resnick, M.D., a partner at SV Health Investors, is a member of our board of directors. SV Health Investors, is a holder of five percent or more of our capital stock.

(3) Consists of (i) 1,808,211 shares of Series A preferred stock held by SV Life Sciences Fund VI, L.P. and (ii) 61,908 shares of Series A preferred stock held by SV Life Sciences Fund VI, Strategic Partners L.P.

(4) Scott G. Requadt, J.D., MBA, a Managing Director at Clarus, is a member of our board of directors. Clarus is a holder of five percent or more of our capital stock.

(5) Citadel Multi-Strategy Equities Master Fund Ltd is a holder of five percent or more of our capital stock

(6) Cormorant Private Healthcare Fund I, LP is a holder of five percent or more of our capital stock

(7) Consists of (i) 4,366,543 shares, all purchased and received by Cormorant Private Healthcare Fund I, L.P. (ii) 1,005,938 shares, all purchased and received by Cormorant Global Healthcare Master Fund, L.P. and (iii) 237,879 shares, all purchased and received by CRMA SPV, L.P.

Strategic and Operational Services

Following the Series Seed preferred stock investment in our company by Atlas Venture X, L.P., or Atlas Venture, we were provided with certain services related to strategic and ordinary course business operations in connection with the incubation of our company during its early stages, including the use of office space provided by the management company for Atlas Venture. During the years ended December 31, 2016 and 2017, we paid to such management company fees in the amount of approximately $78,000 and $15,000, respectively. None of these fees were paid directly or indirectly to Bruce Booth, the chairman of our board of directors and a partner at Atlas Venture. In addition, the fees paid to such management company did not exceed 5% of the consolidated gross revenue of Atlas Venture during any of these fiscal years. Atlas Venture X, L.P. is a beneficial owner of more than 5% of our voting securities.

Agreements with Stockholders

In connection with our Series Seed, Series A and Series B preferred stock financings, we entered into investors' rights, voting and right of first refusal and co-sale agreements containing registration rights, information rights, voting rights and rights of first refusal, among other things, with certain holders of our preferred stock and certain holders of our common stock. These stockholder agreements will terminate upon the closing of this offering, except for the registration rights granted under our investors' rights agreement, as more fully described in "Description of Capital Stock—Registration Rights."

Participation in this Offering

Certain of our existing stockholders, including certain affiliates of our directors, have agreed to purchase an aggregate of approximately $23 million of shares of our common stock in this offering at the initial public offering price.

Indemnification Agreements

In connection with this offering, we intend to enter into agreements to indemnify our directors and executive officers. These agreements will, among other things, require us to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person's status as a member of our board of directors to the maximum extent allowed under Delaware law.

Policies for Approval of Related Party Transactions

Our board of directors reviews and approves transactions with directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party's relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party's relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

In connection with this offering, we have adopted a written related party transactions policy that such transactions must be approved by our audit committee. This policy became effective on the date on which the registration statement of which this prospectus is part is declared effective by the SEC. Pursuant to this policy, the audit committee has the primary responsibility for reviewing and approving or disapproving "related party transactions," which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed $120,000 and in which a related person has or will have a direct or indirect material interest. For purposes of this policy, a related person will be defined as a director, executive officer, nominee for director, or greater than 5% beneficial owner of our common stock, in each case since the beginning of the most recently completed year, and their immediate family members.

PRINCIPAL STOCKHOLDERS

The following table sets forth certain information known to us regarding beneficial ownership of our capital stock as of June 1, 2018, as adjusted to reflect the sale of common stock offered by us in this offering, for:

- each person or group of affiliated persons known by us to be the beneficial owner of more than five percent of our capital stock;
- each of our named executive officers;
- each of our directors; and
- all of our executive officers and directors as a group.

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Under those rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power, and includes securities that the individual or entity has the right to acquire, such as through the exercise of stock options, within 60 days of June 1, 2018. Except as noted by footnote, and subject to community property laws where applicable, we believe, based on the information provided to us, that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

Certain of our existing stockholders, including certain affiliates of our directors, have agreed to purchase an aggregate of approximately $23 million of shares of our common stock in this offering at the initial public offering price. The table below does not give effect to the purchases by such stockholders in this offering.

The percentage of beneficial ownership prior to this offering in the table below is based on 17,901,686 shares of common stock deemed to be outstanding as of June 1, 2018, assuming the conversion of all outstanding shares of our preferred stock upon the closing of this offering into an aggregate of 15,320,213 shares of common stock upon the completion of this offering, and the percentage of beneficial ownership at this offering in the table below is based on 23,149,644 shares of common stock assumed to be outstanding after the closing of the offering. The information in the table below assumes no exercise of the underwriters' option to purchase additional shares.

Except as otherwise noted below, the address for persons listed in the table is c/o AVROBIO, Inc., One Kendall Square, Building 300, Suite 201, Cambridge, MA 02139.

NAME AND ADDRESS OF BENEFICIAL OWNER	NUMBER OF SHARES BENEFICIALLY OWNED PRIOR TO OFFERING	PERCENTAGE OF SHARES BENEFICIALLY OWNED	
		BEFORE OFFERING	AFTER OFFERING
5% Stockholders:			
Atlas Venture Fund X, L.P.(1)	4,756,478	26.57%	20.55%
Clarus Life Sciences III, L.P.(2)	2,962,325	16.55%	12.80%
Affiliates of SV Life Sciences Fund(3)	2,736,027	15.28%	11.82%
Citadel Multi-Strategy Equities Master Fund Ltd(4)	1,357,783	7.58%	5.87%
Affiliates of Cormorant(5)	1,357,781	7.58%	5.87%
Named Executive Officers and Directors:			
Geoff MacKay(6)	687,069	3.81%	2.97%
Katina Dorton	—	—	—
Nerissa Kreher, M.D.(7)	45,054	*	*
Bruce Booth, D.Phil.	—	—	—

NAME AND ADDRESS OF BENEFICIAL OWNER	NUMBER OF SHARES BENEFICIALLY OWNED PRIOR TO OFFERING	PERCENTAGE OF SHARES BENEFICIALLY OWNED	
		BEFORE OFFERING	AFTER OFFERING
Ian T. Clark	—	—	—
Phillip Donenberg	—	—	*
Annalisa Jenkins, M.B.B.S., F.R.C.P.	—	—	—
Christopher Paige, Ph.D.	287,512	1.61%	1.24%
Scott G. Requadt	—	—	—
Joshua Resnick, M.D.	—	—	—
All executive officers and directors as a group (10 persons) (8)	1,019,635	5.64%	4.40%

* Less than 1%

(1) Consists of (i) 806,711 shares of common stock issuable upon conversion of Series Seed preferred stock, (ii) 3,044,578 shares of common stock issuable upon conversion of Series A preferred stock and (iii) 905,189 shares of common stock issuable upon conversion of Series B preferred stock. All shares are held directly by Atlas Venture Fund X, L.P., or Atlas Venture X. Atlas Venture Associates X, L.P., or AVA X LP, is the general partner of Atlas Venture X, and Atlas Venture Associates X, LLC, or AVA X LLC, is the general partner of AVA X LP. Bruce Booth is a member of AVA X LLC and a member of our board of directors. Dr. Booth disclaims beneficial ownership of such shares, except to the extent of his proportionate pecuniary interest therein, if any. The address for Atlas Venture X is 25 First Street, Suite 303, Cambridge, MA 02141.

(2) Consists of (i) 2,283,434 shares of common stock issuable upon conversion of Series A preferred stock and (ii) 678,892 shares of common stock issuable upon conversion of Series B preferred stock. All shares are held directly by Clarus Lifesciences III, L.P., or Clarus. Clarus Ventures III GP, L.P., or the GPLP, as the sole general partner of Clarus, may be deemed to beneficially own certain of the shares held by Clarus. The GPLP disclaims beneficial ownership of all shares held by Clarus in which the GPLP does not have an actual pecuniary interest. Clarus Ventures III, LLC, or the GPLLC, as the sole general partner of the GPLP, may be deemed to beneficially own certain of the shares held by Clarus. The GPLLC disclaims beneficial ownership of all shares held by Clarus in which it does not have an actual pecuniary interest. Each of Nicholas Galakatos, Dennis Henner, Robert Liptak, Scott Requadt, Nicholas Simon, and Kurt Wheeler, as individual managing directors of the GPLLC, may be deemed to beneficially own certain of the shares held of record by Clarus. Each of Messrs. Galakatos, Henner, Liptak, Requadt, Simon and Wheeler disclaims beneficial ownership of all shares held of record by Clarus in which he does not have an actual pecuniary interest. Scott G. Requadt is a member of the GPLLC and a member of our board of directors. Mr. Requadt disclaims beneficial ownership of such shares, except to the extent of his proportionate pecuniary interest therein, if any. The address for the entities is 101 Main Street, Suite 1210, Cambridge, MA 02142.

(3) Consists of (i) 2,207,843 shares of common stock issuable upon conversion of shares of Series A preferred stock held of record by SV Life Sciences Fund VI, L.P., (ii) 75,590 shares of common stock issuable upon conversion of shares of Series A preferred stock held of record by SV Life Sciences Fund VI, Strategic Partners L.P., (iii) 437,611 shares of common stock issuable upon conversion of shares of Series B preferred stock held of record by SV Life Sciences Fund VI, L.P. and (iv) 14,983 shares of common stock issuable upon conversion of shares of Series B preferred stock held of record by SV Life Sciences Fund VI, Strategic Partners L.P. SV Health Investors, LLC is the Manager of SV Life Sciences Fund VI, L.P. and SV Life Sciences Fund VI Strategic Partners, L.P. SV Life Sciences Fund VI (GP), LP, or SV Fund VI GP, is the general partner of SV Life Sciences Fund VI, L.P. and SV Life Sciences Fund VI Strategic Partners, L.P. The general partner of SV Fund VI GP is SVLSF VI, LLC. The members of the investment committee of SVLSF VI, LLC are Kate Bingham, Thomas Flynn, James Garvey, Eugene D. Hill, III, Paul LaViolette, and Michael Ross. Each of SV Fund VI GP, SVLSF VI, LLC and the SVLSF VI, LLC investment committee disclaims beneficial ownership of the shares owned directly by SV Life Sciences Fund VI, L.P. and SV Life Sciences Fund VI Strategic Partners, L.P. except to the extent of any pecuniary interest therein. The address

for each of the entities and individuals listed above is One Boston Place, Suite 3900, 201 Washington Street, Boston, Massachusetts 02108.

(4) Consists of 1,357,783 shares of common stock issuable upon conversion of Series B preferred stock. The address for Citadel Multi-Strategy Equities Master Fund Ltd. is c/o Citadel Advisors LLC, 601 Lexington Avenue, New York, NY 10022.

(5) Consists of (i) 1,056,762 shares of common stock issuable upon conversion of shares of Series B preferred stock held of record by Cormorant Private Healthcare Fund I, LP, or Cormorant Private Fund, (ii) 243,450 shares of common stock issuable upon conversion of shares of Series B preferred stock held of record by Cormorant Global Healthcare Master Fund, LP, or Cormorant Master Fund and (iii) 57,569 shares of common stock issuable upon conversion of shares of Series B preferred stock held of record by CRMA SPV, L.P., or CRMA. The sole general partner of Cormorant Private Fund is Cormorant Private Healthcare GP, LLC and the sole general partner of Cormorant Master Fund is Cormorant Global Healthcare GP, LLC, and together Cormorant GP. Bihua Chen is the sole managing member of Cormorant GP, and may be deemed to have sole voting and investment power of the securities held by the Cormorant Private Fund and the Cormorant Master Fund. The sole investment manager of CRMA is Cormorant Asset Management, LLC, or the Manager. Bihua Chen is the sole managing member of the Manager, and may be deemed to have sole voting and investment power of the securities held by CRMA. The address of the Cormorant Private Fund, the Cormorant Master Fund and CRMA is 200 Clarendon Street, 52nd Floor, Boston, MA 02116.

(6) Consists of (i) 423,523 shares of common stock, (ii) 72,604 shares of common stock held by Mac MacKay, (iii) 72,604 shares of common stock held by Kali MacKay and (iv) 118,338 shares of common stock issuable upon exercise of options within 60 days of June 1, 2018. Mr. MacKay is the father of Mac MacKay and Kali MacKay. Mr. MacKay may be deemed to have voting and investment power over shares held by Mac MacKay and Kali MacKay.

(7) Includes 45,054 shares of common stock issuable upon exercise of options within 60 days of June 1, 2018.

(8) Includes an aggregate of 139,887 shares issuable upon exercise of stock options within 60 days of June 1, 2018 held by our executive officers and directors as a group.

DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our amended and restated certificate of incorporation and amended and restated bylaws, which will be effective upon the closing of this offering. The descriptions of the common stock and preferred stock give effect to changes to our capital structure that will occur upon the completion of this offering. We refer in this section to our amended and restated certificate of incorporation as our certificate of incorporation, and we refer to our amended and restated bylaws as our bylaws.

General

Upon completion of this offering, our authorized capital stock will consist of 150,000,000 shares of common stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share, all of which shares of preferred stock will be undesignated.

As of March 31, 2018, 2,581,474 shares of our common stock, 3,333,333 shares of Series Seed preferred stock, 31,450,499 shares of Series A preferred stock and 28,519,322 shares of Series B preferred stock were outstanding and held by 24 stockholders of record.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred Stock

Upon the completion of this offering, all outstanding shares of our preferred stock will be converted into shares of our common stock. Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Stock Options

As of March 31, 2018, there were outstanding options to purchase an aggregate of 1,788,750 shares of our common stock.

Registration Rights

Upon the completion of this offering, the holders of 15,320,213 shares of our common stock, including those issuable upon the conversion of preferred stock, will be entitled to rights with respect to the registration of these securities under the Securities Act. These rights are provided under the terms of an investors' rights agreement between us and holders of our preferred stock. The investors' rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under this agreement will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand Registration Rights

Beginning 180 days after the effective date of this registration statement, the holders of 15,320,213 shares of our common stock, including those issuable upon the conversion of preferred stock upon closing of this offering, are entitled to demand registration rights. Under the terms of the investors' rights agreement, we will be required, upon the written request of holders of at least 50% of these securities that would result in an aggregate offering price of at least $10.0 million, to file a registration statement and use commercially reasonable efforts to effect the registration of all or a portion of these shares for public resale. We are required to effect only two registrations pursuant to this provision of the investors' rights agreement.

Short-Form Registration Rights

Pursuant to the investors' rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of holders of at least 25% of these securities at an aggregate offer price of at least $3.0 million, we will be required to use commercially reasonable efforts to effect a registration of such shares. We are required to effect only two registrations in any twelve month period pursuant to this provision of the investors' rights agreement. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Piggyback Registration Rights

Pursuant to the investors' rights agreement, if we register any of our securities either for our own account or for the account of other security holders, the holders of these shares are entitled to include their shares in the registration. Subject to certain exceptions contained in the investors' rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering.

Indemnification

Our investors' rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

Expiration of Registration Rights

The demand registration rights and short form registration rights granted under the investors' rights agreement will terminate on the earliest of (i) a deemed liquidation event, as defined in the investors' rights agreement, (ii) the fifth anniversary of the completion of this offering and (iii) at such time after this offering when the holders' shares may be sold without restriction pursuant to Rule 144 within a three month period.

Anti-Takeover Effects of our Certificate of Incorporation and Bylaws and Delaware Law

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering will include a number of provisions that may have the effect of

delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

Our amended and restated certificate of incorporation that will become effective upon the closing of this offering will provide for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our amended and restated certificate of incorporation will also provide that directors may be removed only for cause and then only by the affirmative vote of the holders of two-thirds or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No Written Consent of Stockholders

Our amended and restated certificate of incorporation that will become effective upon the closing of this offering will provide that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of Stockholders

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering will provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our amended and restated bylaws will limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our amended and restated bylaws that will become effective upon the closing of this offering will establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our bylaws specify the requirements as to form and content of all stockholders' notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

Amendment to Certificate of Incorporation and Bylaws

Any amendment of our amended and restated certificate of incorporation that will become effective upon the closing of this offering must first be approved by a majority of our board of directors, and if required by law or our certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition, limitation of

liability and the amendment of our bylaws and certificate of incorporation must be approved by not less than two-thirds of the outstanding shares entitled to vote on the amendment, and not less than two-thirds of the outstanding shares of each class entitled to vote thereon as a class. Our bylaws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the bylaws; and may also be amended by the affirmative vote of at least two-thirds of the outstanding shares entitled to vote on the amendment, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated Preferred Stock

Our amended and restated certificate of incorporation that will become effective upon the closing of this offering will provide for 10,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Choice of Forum

Our amended and restated bylaws that will become effective upon the closing of this offering will provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of or based on a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (3) any action asserting a claim against us or any of our current or former directors, officers, employees or stockholders arising pursuant to any provision of the Delaware General Corporation Law or our certificate of incorporation or bylaws; or (4) any action asserting a claim governed by the internal affairs doctrine. Our amended and restated bylaws also provide that any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock will be deemed to have notice of and to have consented to this choice of forum provision.

In addition, our amended and restated bylaws that will become effective upon the closing of this offering will contain a provision by virtue of which unless we consent in writing to the selection of an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. If any action the subject matter of which is within the scope of the preceding sentence is filed in a court other than the United States District Court for the District of Massachusetts, the plaintiff or plaintiffs shall be deemed by this provision of our amended and restated bylaws (i) to have consented to removal of the action by us to the United States District Court for the District of Massachusetts, in the case of an action filed in a state court, and (ii) to have consented to transfer of the action to the United States District Court for the District of Massachusetts. We have chosen the United States Court for the District of Massachusetts as the exclusive forum for such causes of action because our principal executive offices are located in Cambridge, Massachusetts. Some companies that have adopted similar federal district court forum selection provisions are currently subject to a suit in the Court of Chancery of the State of Delaware by stockholders who assert that the federal district court forum selection provision is not enforceable. We recognize that the federal district court forum selection clause may impose additional litigation costs on stockholders who assert the provision

is not enforceable and may impose more general additional litigation costs in pursuing any such claims, particularly if the stockholders do not reside in or near the Commonwealth of Massachusetts. Additionally, the forum selection clauses in our amended and restated bylaws may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us. Alternatively, if the federal district court forum selection provision is found inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have an adverse effect on our business, financial condition or results of operations. The U.S. District Court in Massachusetts may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Nasdaq Global Select Market Listing

We have been approved to list our common stock on The Nasdaq Global Select Market under the trading symbol “AVRO.”

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be Computershare Trust Company, N.A. The transfer agent and registrar’s address is 250 Royall Street, Canton, Massachusetts 02021, and its telephone number is (800) 962-4284.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for shares of our common stock. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of March 31, 2018, upon the completion of this offering, 23,149,645 shares of our common stock will be outstanding, assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options. Of the outstanding shares, all of the shares sold in this offering will be freely tradable, except that any shares held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below, and shares of our common stock are restricted shares of common stock subject to time-based vesting terms. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, summarized below.

Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Securities Exchange Act of 1934, as amended, or the Exchange Act, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately 231,496 shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares, based on the number of shares outstanding as of March 31, 2018; or
- the average weekly trading volume of our common stock on The Nasdaq Global Select Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares.

However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under "Underwriters" included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-Up Agreements

In connection with this offering, we and all of our directors, executive officers and substantially all of the holders of our stock and stock options have signed a lock-up agreement that prevents them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of Morgan Stanley & Co. LLC and Cowen and Company, LLC, subject to certain exceptions. See the section entitled “Underwriters” appearing elsewhere in this prospectus for more information.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled “Description of Capital Stock—Registration Rights” appearing elsewhere in this prospectus for more information.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above. As of June 1, 2018, we estimate that such registration statement on Form S-8 will cover approximately 2,628,250 shares.

10b5-1 Plans

After the offering, certain of our employees, including our executive officers and/or directors may enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Sales under these trading plans would not be permitted until the expiration of the lock-up agreements relating to the offering described above.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following discussion is a summary of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a foreign corporation or any other foreign organization taxable as a corporation for U.S. federal income tax purposes; or
- a foreign estate or trust, the income of which is not subject to U.S. federal income tax on a net income basis.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset within the meaning of Section 1221 of the Code, generally property held for investment.

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of any U.S. federal tax other than the income tax, U.S. state, local or non-U.S. taxes, the alternative minimum tax, rules regarding qualified small business stock within the meaning of Section 1202 of the Code or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;
- "qualified foreign pension funds," or entities wholly owned by a "qualified foreign pension fund";
- persons that have a functional currency other than the U.S. dollar;

- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on Our Common Stock

Distributions, if any, on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "Gain on Sale or Other Taxable Disposition of Our Common Stock." Any such distributions will also be subject to the discussions below under the sections titled "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA."

Subject to the discussion in the following two paragraphs in this section, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) to the applicable withholding agent and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Sale or Other Taxable Disposition of Our Common Stock

Subject to the discussions below under "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA," a non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base

maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on Our Common Stock" also may apply;

- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been, at any time during the five-year period preceding such sale of other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above.

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on Our Common Stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

Withholding and Information Reporting Requirements—FATCA

The Foreign Account Tax Compliance Act, or FATCA, generally imposes a U.S. federal withholding tax at a rate of 30% on payments of dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA currently applies to payments of dividends on our common stock, but will only apply to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2018. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

UNDERWRITERS

Under the terms and subject to the conditions in an underwriting agreement dated the date of this prospectus, the underwriters named below, for whom Morgan Stanley & Co. LLC, Cowen and Company, LLC and Wells Fargo Securities, LLC are acting as representatives, have severally agreed to purchase, and we have agreed to sell to them, severally, the number of shares indicated below:

Name	Number of Shares
Morgan Stanley & Co. LLC	2,361,581
Cowen and Company, LLC	1,574,387
Wells Fargo Securities, LLC	787,194
Wedbush Securities Inc.	524,796
Total:	5,247,958

The underwriters and the representatives are collectively referred to as the “underwriters” and the “representatives,” respectively. The underwriters are offering the shares of common stock subject to their acceptance of the shares from us and subject to prior sale. The underwriting agreement provides that the obligations of the several underwriters to pay for and accept delivery of the shares of common stock offered by this prospectus are subject to the approval of certain legal matters by their counsel and to certain other conditions. The underwriters are obligated to take and pay for all of the shares of common stock offered by this prospectus if any such shares are taken. However, the underwriters are not required to take or pay for the shares covered by the underwriters’ over-allotment option described below.

The underwriters initially propose to offer part of the shares of common stock directly to the public at the offering price listed on the cover page of this prospectus and part to certain dealers at a price that represents a concession not in excess of $0.798 per share under the public offering price. After the initial offering of the shares of common stock, the offering price and other selling terms may from time to time be varied by the representatives.

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to 787,193 additional shares of common stock at the public offering price listed on the cover page of this prospectus, less underwriting discounts and commissions. The underwriters may exercise this option solely for the purpose of covering over-allotments, if any, made in connection with the offering of the shares of common stock offered by this prospectus. To the extent the option is exercised, each underwriter will become obligated, subject to certain conditions, to purchase about the same percentage of the additional shares of common stock as the number listed next to the underwriter’s name in the preceding table bears to the total number of shares of common stock listed next to the names of all underwriters in the preceding table.

The following table shows the per share and total public offering price, underwriting discounts and commissions, and proceeds before expenses to us. These amounts are shown assuming both no exercise and full exercise of the underwriters’ option to purchase up to an additional 787,193 shares of common stock.

	Per Share	Total	
		No Exercise	Full Exercise
Public offering price	$19.00	$99,711,202	$114,667,869
Underwriting discounts and commissions to be paid by us:	$ 1.33	$ 6,979,784	$ 8,026,751
Proceeds, before expenses, to us	$17.67	$92,731,418	$106,641,118

The estimated offering expenses payable by us, exclusive of the underwriting discounts and commissions, are approximately $2,276,000. We have agreed to reimburse the underwriters for expenses relating to clearance of this offering with the Financial Industry Regulatory Authority up to $30,000.

The underwriters have informed us that they do not intend sales to discretionary accounts to exceed 5% of the total number of shares of common stock offered by them.

We have been approved to list our common stock on The Nasdaq Global Select Market under the symbol “AVRO.”

We and all of our directors and officers and the holders of substantially all of our outstanding stock and stock options have agreed that, without the prior written consent of Morgan Stanley & Co. LLC and Cowen and Company, LLC, on behalf of the underwriters, we and they will not, during the period ending 180 days after the date of this prospectus (the “restricted period”):

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock;
- file any registration statement with the Securities and Exchange Commission relating to the offering of any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock; or
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock.

whether any such transaction described above is to be settled by delivery of common stock or such other securities, in cash or otherwise. In addition, we and each such person agrees that, without the prior written consent of Morgan Stanley & Co. LLC and Cowen and Company, LLC, on behalf of the underwriters, we or such other person will not, during the restricted period, make any demand for, or exercise any right with respect to, the registration of any shares of common stock or any security convertible into or exercisable or exchangeable for common stock.

The restrictions described in the immediately preceding paragraph to do not apply to:

- transactions relating to shares of common stock or other securities acquired in this offering or acquired in open market transactions after this offering
- transfers of shares of common stock or any security convertible into common stock as a bona fide gift;
- distributions of shares of common stock or any security convertible into common stock to limited partners or stockholders of the signatory;
- the establishment of a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, provided that such plan does not provide for the transfer of shares of common stock during the restricted period;
- transfers or dispositions of shares of common stock or other securities to any member of the immediate family of the signatory or any trust for the direct or indirect benefit of the signatory or the immediate family of the signatory in a transaction not involving a disposition for value;
- transfers or dispositions of shares of common stock or other securities to any corporation, partnership, limited liability company or other entity controlled or managed by the signatory, in a transaction not involving a disposition for value;
- transfers or dispositions of shares for common stock or other securities (i) by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the signatory upon the death of the signatory, or (ii) by operation of law pursuant to a domestic order or negotiated divorce settlement;
- transfers or dispositions of common stock or any security convertible into or exercisable or exchangeable for common stock to us pursuant to any contractual arrangement in effect prior to the

date of this prospectus and disclosed to Morgan Stanley & Co. LLC and Cowen and Company, LLC, that provides for the repurchase of common stock or other securities by us or in connection with the termination of employment with or service to us, provided that the repurchase price for any such shares of common stock or other securities shall not exceed the original purchase price (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization) paid to us for such shares or securities, and, provided further that any public announcement or public filing under Section 16(a) of the Exchange Act required to be made during the restricted period in connection with such transfer or disposition shall clearly indicate in the footnotes thereto or comments section thereof that such transfer or disposition was made solely to us pursuant to the circumstances described above;

- the conversion of any convertible preferred stock described in this prospectus and outstanding as of the date of this prospectus into, or the exercise of any option or warrant described in the prospectus and outstanding as of the date hereof for, shares of common stock, provided that any such shares of common stock received by the signatory shall be subject to the terms of the lock-up agreement; provided, further, that any public filing or public announcement under Section 16(a) of the Exchange Act required during the restricted period in connection with the conversion of such preferred stock or the exercise of such stock option or warrant shall clearly indicate in the footnotes thereto or comments section thereof that the filing relates to the conversion of preferred stock or the exercise of a stock option or warrant, as the case may be, that no shares of common stock were sold by the reporting person and that the shares of common stock received upon exercise of the stock option or warrant are subject to a lock-up agreement with the underwriters of this offering;
- transfers or dispositions of title to (but not beneficial ownership of) shares of common stock or other securities to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under any of the foregoing clauses; provided that any such shares of common stock or other securities shall remain subject to the terms of the lock-up agreement; or
- transfers or dispositions of shares of common stock or such other securities pursuant to a bona fide tender offer for shares of our capital stock, merger, consolidation or other similar transaction made to all holders of our securities involving a change of control of us that has been approved by our board of directors, provided that, in the event that the change of control transaction is not consummated, this clause shall not be applicable to the lock-up signatory's shares and other securities shall remain subject to the restrictions contained in the lock-up agreement;

provided that, in the case of any transfer or distribution as described in the second, third, fifth, sixth or seventh bullet point above, the transferee or distributee shall agree to be subject to the restrictions described in the immediately preceding paragraph and (ii) in the case of any transfer or distribution described in the first, second, third, fourth, fifth, sixth, seventh or tenth bullet point above, no public announcement or public filing under Section 16(a) of the Exchange Act relating to such transfer or distribution shall be required or shall be voluntarily made during the restricted period.

In addition, the restrictions described in the paragraph above relating to us do not apply to:

- the shares to be sold in this offering;
- our issuance of shares of common stock upon the exercise of an option or warrant or the conversion of a security outstanding on the date of this prospectus pursuant to stock plans disclosed in this prospectus; or
- the establishment of a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, provided that such plan does not provide for the transfer of shares of common stock during the restricted period and to the extent a public announcement or filing under the Exchange Act is required of or voluntarily made by the Company regarding the establishment of such plan, such announcement or filing shall include a statement to the effect that no transfer of shares of common stock may be made under such plan during the restricted period;

Morgan Stanley & Co. LLC and Cowen and Company, LLC, in their sole discretion, may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time.

In order to facilitate the offering of the common stock, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of the common stock. Specifically, the underwriters may sell more shares than they are obligated to purchase under the underwriting agreement, creating a short position. A short sale is covered if the short position is no greater than the number of shares available for purchase by the underwriters under the over-allotment option. The underwriters can close out a covered short sale by exercising the over-allotment option or purchasing shares in the open market. In determining the source of shares to close out a covered short sale, the underwriters will consider, among other things, the open market price of shares compared to the price available under the over-allotment option. The underwriters may also sell shares in excess of the over-allotment option, creating a naked short position. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in this offering. As an additional means of facilitating this offering, the underwriters may bid for, and purchase, shares of common stock in the open market to stabilize the price of the common stock. These activities may raise or maintain the market price of the common stock above independent market levels or prevent or retard a decline in the market price of the common stock. The underwriters are not required to engage in these activities and may end any of these activities at any time.

We and the underwriters have agreed to indemnify each other against certain liabilities, including liabilities under the Securities Act.

A prospectus in electronic format may be made available on websites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The representatives may agree to allocate a number of shares of common stock to underwriters for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters that may make Internet distributions on the same basis as other allocations.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. Certain of the underwriters and their respective affiliates may, from time to time, perform various financial advisory and investment banking services for us, for which they will receive customary fees and expenses.

In addition, in the ordinary course of their various business activities, the underwriters and their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers and may at any time hold long and short positions in such securities and instruments. Such investment and securities activities may involve our securities and instruments. The underwriters and their respective affiliates may also make investment recommendations or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long or short positions in such securities and instruments.

Pricing of the Offering

Prior to this offering, there has been no public market for our common stock. The initial public offering was determined by negotiations between us and the representatives. Among the factors considered in determining the initial public offering price were our future prospects and those of our industry in general, our sales, earnings and certain other financial and operating information in recent periods, and the price-earnings ratios, price-sales

ratios, market prices of securities, and certain financial and operating information of companies engaged in activities similar to ours.

Selling Restrictions

Canada

Shares of our common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration Requirements, Exemptions and Ongoing Registrant Obligations*. Any resale of shares of our common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State") an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

Each underwriter has represented and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000 ("FSMA")) received by it in connection with the issue or sale of the shares of our common stock in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares of our common stock in, from or otherwise involving the United Kingdom.

LEGAL MATTERS

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters relating to this offering will be passed upon for the underwriters by Ropes & Gray LLP, Boston, Massachusetts.

EXPERTS

The consolidated financial statements of AVROBIO, Inc. at December 31, 2016 and 2017, and for the years then ended, appearing in this prospectus and registration statement have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 (File Number 333-225213) under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon the completion of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facility at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. We also maintain a website at www.avrobio.com. Upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and any amendments to those reports, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

You may also obtain copies of the documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities.

AVROBIO, INC.
INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Index to Consolidated Financial Statements as of December 31, 2016 and 2017 and for the Years Ended December 31, 2016 and 2017

Index to Unaudited Interim Condensed Consolidated Financial Statements as of December 31, 2017 and March 31, 2018 and for the Three Month Periods Ended March 31, 2017 and 2018

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors of AVROBIO, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of AVROBIO, Inc. (the Company) as of December 31, 2016 and 2017, the related consolidated statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholder's deficit and cash flows for the years then ended, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2016 and 2017, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2018.
Boston, Massachusetts
April 6, 2018, except for Note 17(b),
as to which the date is June 11, 2018

AVROBIO, INC.
CONSOLIDATED BALANCE SHEETS
(amounts in thousands, except share and per share data)

	December 31, 2016	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 5,357	$ 5,963
Prepaid expenses and other current assets	19	345
Total current assets	5,376	6,308
Property and equipment, net	—	349
Other assets	24	365
Total assets	$ 5,400	$ 7,022
Liabilities, redeemable convertible preferred stock and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 340	$ 527
Accrued expenses and other current liabilities	551	2,098
Total current liabilities	891	2,625
Warrant to purchase redeemable convertible preferred stock	—	35
Derivative liability	88	371
Deferred rent, net of current portion	—	126
Other long-term liability	—	500
Total liabilities	979	3,657
Commitments and contingencies (Note 14)		
Redeemable convertible preferred stock (Note 8)	9,000	26,500
Stockholders' (deficit) equity:		
Common stock, $0.0001 par value; 40,000,000 and 51,000,000 shares authorized as of December 31, 2016 and 2017, respectively; 2,581,474 shares issued as of December 31, 2016 and 2017; 2,172,068 and 2,305,173 shares outstanding as of December 31, 2016 and 2017, respectively	—	—
Additional paid-in capital	247	339
Accumulated deficit	(4,826)	(23,474)
Total stockholders' deficit	(4,579)	(23,135)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	$ 5,400	$ 7,022

The accompanying notes are an integral part of these consolidated financial statements.

AVROBIO, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(amounts in thousands, except share and per share data)

	Year Ended December 31,	
	2016	2017
Operating expenses:		
Research and development	$ 2,663	$ 15,191
General and administrative	1,962	3,195
Total operating expenses	4,625	18,386
Loss from operations	(4,625)	(18,386)
Other income (expense):		
Interest income	6	57
Change in fair value of preferred stock warrant liability	—	(17)
Change in fair value of derivative liability	(39)	(283)
Other expense	(6)	(19)
Total other expense, net	(39)	(262)
Net loss	$ (4,664)	$ (18,648)
Comprehensive loss	$ (4,664)	$ (18,648)
Reconciliation of net loss to net loss attributable to common stockholders:		
Net loss	$ (4,664)	$ (18,648)
Accretion of issuance costs on redeemable convertible preferred stock	(305)	(85)
Net loss attributable to common stockholders—basic and diluted	$ (4,969)	$ (18,733)
Net loss per share attributable to common stockholders—basic and diluted (Note 13)	$ (2.44)	$ (8.38)
Weighted-average number of common shares used in computing net loss per share attributable to common stockholders—basic and diluted	2,038,025	2,235,865
Pro forma net loss per share attributable to common stockholders—basic and diluted (unaudited) (Note 2)		$ (2.69)
Pro forma weighted-average number of common shares used in computing pro forma net loss per share attributable to common stockholders—basic and diluted (unaudited)		6,922,173

The accompanying notes are an integral part of these consolidated financial statements.

AVROBIO, INC.
CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(amounts in thousands, except share data)

	Series Seed Redeemable Convertible Preferred Stock		Series A Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance as of December 31, 2015	—	$ —	—	$ —	1,258,467	$ —	$ —	$ (162)	$ (162)
Issuance of series Seed redeemable convertible preferred stock, net of issuance costs of $102	3,333,333	1,398	—	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	1,161,665	—	480	—	480
Modification of founders common stock to include certain time-based vesting restricitions	—	—	—	—	(330,750)	—	—	—	—
Vesting of restricted stock awards	—	—	—	—	82,686	—	—	—	—
Issuance of series A redeemable convertible preferred stock, net of issuance costs of $203	—	—	5,714,286	7,297	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	72	—	72
Accretion of issuance costs related to redeemable convertible preferred stock	—	102	—	203	—	—	(305)	—	(305)
Net loss	—	—	—	—	—	—	—	(4,664)	(4,664)
Balance as of December 31, 2016	3,333,333	1,500	5,714,286	7,500	2,172,068	—	247	(4,826)	(4,579)
Issuance of series A redeemable convertible preferred stock dividend	—	—	3,720,864	—	—	—	—	—	—
Issuance of series A redeemable convertible preferred stock, net of issuance costs of $85	—	—	22,015,349	17,415	—	—	—	—	—
Vesting of restricted stock awards	—	—	—	—	133,105	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	177	—	177
Accretion of issuance costs related to redeemable convertible preferred stock	—	—	—	85	—	—	(85)	—	(85)
Net loss	—	—	—	—	—	—	—	(18,648)	(18,648)
Balance as of December 31, 2017	3,333,333	$ 1,500	31,450,499	$ 25,000	2,305,173	$ —	$ 339	$ (23,474)	$ (23,135)

The accompanying notes are an integral part of these consolidated financial statements.

AVROBIO, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(amounts in thousands)

	Year Ended December 31,	
	2016	2017
Cash flows from operating activities:		
Net loss	$(4,664)	$(18,648)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	72	177
Depreciation and amortization expense	—	45
Amortization of deferred offering costs	—	24
Non-cash license expense	480	—
Non-cash research and development expense related to derivative liability	49	—
Deferred rent expense	—	134
Change in fair value of preferred stock warrant liability	—	17
Change in fair value of derivative liability	39	283
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(18)	(326)
Other assets	—	(218)
Accounts payable	177	176
Accrued expenses and other current liabilities	551	1,454
Other long-term liability	—	500
Net cash used in operating activities	(3,314)	(16,382)
Cash flows from investing activities:		
Purchases of property and equipment	—	(383)
Changes in restricted cash	(24)	—
Net cash used in investing activities	(24)	(383)
Cash flows from financing activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	8,695	17,415
Payments of issuance costs on debt facility	—	(44)
Net cash provided by financing activities	8,695	17,371
Net increase in cash and cash equivalents	5,357	606
Cash and cash equivalents at beginning of period	—	5,357
Cash and cash equivalents at end of period	$ 5,357	$ 5,963
Supplemental disclosure of non-cash investing and financing activities:		
Purchases of property and equipment included in accounts payable	$ —	$ 11
Issuance of warrants associated with debt facility	$ —	$ 18
Deferred offering costs included in accrued expenses	$ —	$ 85
Accretion of issuance costs related to redeemable convertible preferred stock	$ 305	$ 85

The accompanying notes are an integral part of these consolidated financial statements.

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Years ended December 31, 2016 and 2017
(amounts in thousands, except share and per share data)

1. Nature of the Business

AVROBIO, Inc. (the "Company" or "AVROBIO") is a clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral gene therapies to treat rare diseases following a single dose.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Through December 31, 2017, the Company has funded its operations primarily with proceeds from the sale of series Seed redeemable convertible preferred stock (the "Series Seed Preferred Stock") and series A redeemable convertible preferred stock (the "Series A Preferred Stock"), (the Series Seed Preferred Stock and the Series A Preferred Stock are collectively referred to as the "Preferred Stock"). The Company has incurred recurring losses since its inception, including net losses of $4,664 and $18,648 for the years ended December 31, 2016 and 2017, respectively. In addition, as of December 31, 2017, the Company had an accumulated deficit of $23,475. The Company expects to continue to generate operating losses for the near future. The future viability of the Company is dependent on its ability to raise additional capital to finance its operations. The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurance that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

The Company believes the cash and cash equivalents on hand as of December 31, 2017 of $5,963, together with the $60,500 of gross cash proceeds received from the Company's sale of series B redeemable convertible preferred stock (the "Series B Preferred Stock") in January 2018 (see Note 17) will be sufficient to fund its operations and capital expenditure requirements through at least April 6, 2019.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Principles of Consolidation

The accompanying consolidated financial statements include the accounts of AVROBIO, Inc. and its wholly owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

2. Summary of Significant Accounting Policies (continued)

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual for research and development expenses, stock-based compensation expense, the valuation of equity and derivative instruments and the recoverability of the Company's net deferred tax assets and related valuation allowance. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from those estimates.

Unaudited Pro Forma Information

In the accompanying consolidated statements of operations and comprehensive loss, the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2017 has been computed using the weighted-average number of common shares outstanding after giving pro forma effect to the conversion of all outstanding shares of redeemable convertible preferred stock into 8,418,149 shares of common stock and the conversion of the outstanding warrant to purchase shares of redeemable convertible preferred stock as of December 31, 2017 into a warrant to purchase shares of common stock, as if such conversion had occurred at the beginning of the period presented or the date of original issuance, whichever is later. Accordingly, the effect of the accretion to redemption value of the redeemable convertible preferred stock has been excluded from the determination of pro forma basic and diluted loss per share attributable to common stockholders. Additionally, the changes in the fair value of the warrant to purchase redeemable convertible preferred stock has been excluded from the determination of pro forma basic and diluted net loss per share attributable to common stockholders as these instruments are not required be recorded at fair value once it become a warrant to purchase common stock.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in interest-bearing money market accounts. Cash equivalents are carried at cost, which approximates their fair market value.

Restricted Cash

As of December 31, 2016 and 2017, restricted cash consisted of $24 used to secure a letter of credit for the benefit of the landlord in connection with the Company's lease agreement (Note 14). These amounts are classified as other assets in the Company's consolidated balance sheets.

Concentrations of Credit Risk

The Company has no significant off-balance sheet risk, such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and restricted cash. Periodically, the Company maintains deposits in accredited financial institutions in excess of federally insured limits. The

2. Summary of Significant Accounting Policies (continued)

Company deposits its cash and cash equivalents in financial institutions that it believes have high credit quality and has not experienced any losses on such accounts and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

Deferred Issuance Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred issuance costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds generated as a result of the offering. Should the planned equity financing be abandoned, the deferred issuance costs will be expensed immediately as a charge to operating expenses in the consolidated statements of operations. As of December 31, 2017, the Company recorded deferred issuance costs of $85 within other assets on the consolidated balance sheet related to the Series B Preferred Stock offering which was consummated in January 2018 (Note 17). There were no amounts deferred as of December 31, 2016.

Property and Equipment

Property and equipment are recorded at cost. Depreciation and amortization is calculated using the straight-line method over the following estimated useful lives of the assets:

	Estimated Useful Life
Laboratory and office equipment	5 years
Computer equipment and software	2 years
Leasehold improvements	Lesser of lease term or 10 years

Upon disposal, retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the results of operations. Expenditures for repairs and maintenance that do not improve or extend the lives of the respective assets are charged to expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses on long-lived assets.

2. Summary of Significant Accounting Policies (continued)

Fair Value Measurements

Certain assets and liabilities of the Company are carried at fair value under GAAP (see Note 3). Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

Segment Information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision making group, in making decisions on how to allocate resources and assess performance. The Company's chief operating decision maker is the chief executive officer ("CEO"). The Company and the CEO view the Company's operations and manage its business as one operating segment. All material long-lived assets of the Company reside in the United States.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including salaries, stock-based compensation and benefits, facilities costs, depreciation, third-party license fees, and external costs of outside vendors engaged to conduct preclinical development activities and clinical trials as well as to manufacture research and development materials. Non-refundable prepayments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as an expense as the goods are delivered or the related services are performed or until it is no longer expected that the goods will be delivered or the services rendered.

The Company has entered into various research and development related contracts with parties both inside and outside of the United States. The payments to these agreements are recorded as research and development expenses as incurred. The Company records accrued liabilities for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

2. Summary of Significant Accounting Policies (continued)

Stock-Based Compensation

For stock-based awards issued to employees and members of the Company's board of directors (the "Board") for their services on the Board, the Company measures the estimated fair value of the stock-based award on the date of grant and recognizes compensation expense for those awards over the requisite service period, which is generally the vesting period of the respective award. The Company issues stock-based awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company has not issued any stock-based awards with performance- or market-based vesting conditions. The Company accounts for forfeitures as they occur.

Stock-based awards issued to non-employees are recorded at their fair values, and are periodically revalued as the awards vest and are recognized as expense over the related service period. For stock-based awards granted to non-employees subject to graded vesting that only contain service conditions, the Company has elected to recognize stock-based compensation expense using the straight-line recognition method.

The Company classifies stock-based compensation expense in its consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's cash compensation costs are classified.

Given the absence of an active market for the Company's common stock, the Company and the Board, the members of which the Company believes have extensive business, finance, and venture capital experience, were required to estimate the fair value of the Company's common stock at the time of each grant of a stock-based award. The Company and the Board determined the estimated fair value of the Company's equity instruments based on a number of factors, including external market conditions affecting the biotechnology industry sector. The Company and the Board utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its common stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors in determining the value of the Company's common stock at each grant date, including: (1) prices paid for the Company's redeemable convertible preferred stock, which the Company had sold to outside investors in arm's-length transactions, and the rights, preferences, and privileges of the Company's redeemable convertible preferred stock and common stock; (2) valuations performed by an independent valuation specialist; (3) the Company's stage of development; (4) the fact that the grants of stock-based awards involved illiquid securities in a private company; and (5) the likelihood of achieving a liquidity event for the common stock underlying the stock-based awards, such as an IPO or sale of the Company, given prevailing market conditions.

The fair value of each stock option award is estimated on the date of grant using the Black-Scholes option pricing model. As there is no public market for its common stock, the Company determined the volatility for awards granted based on an analysis of reported data for a group of guideline companies that issued options with substantially similar terms. The expected volatility has been determined using a weighted-average of the historical volatility measures of this group of guideline companies. The Company expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The Company has not paid, and does not anticipate paying, cash dividends on its common stock; therefore, the expected dividend yield is assumed to be zero.

2. Summary of Significant Accounting Policies (continued)

See Note 10 for the assumptions used by the Company in determining the grant date fair value of stock-based awards granted, as well as a summary of the stock-based award activity under the Company's stock-based compensation plan for the year ended December 31, 2017.

Warrant to Purchase Preferred Stock

The Company classifies the warrant for the purchase of shares of its redeemable convertible preferred stock (see Note 7) as a liability on its consolidated balance sheets as the warrant is a free-standing financial instrument that may require the Company to transfer assets upon exercise. The preferred stock warrant liability was initially recorded at fair value upon the date of issuance and is subsequently remeasured to fair value at each reporting date. Changes in the fair value of the warrant to purchase preferred stock are recognized as a component of other income (expense), net in the consolidated statements of operations and comprehensive loss. Changes in the fair value of the warrant to purchase preferred stock will continue to be recognized until the warrant is exercised, expires or qualifies for equity classification.

The Company utilizes the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value the warrant. The Company assesses these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying redeemable convertible preferred stock issuable upon exercise of the warrant, the remaining contractual term of the warrant, the risk-free interest rate, the expected dividend yield and the expected volatility of the price of the underlying redeemable convertible preferred stock.

Comprehensive Income (Loss)

Comprehensive income (loss) is defined as the change in stockholders' equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive income (loss) includes net income (loss) as well as other changes in stockholders' (deficit) equity which includes certain changes in equity that are excluded from net income (loss). Comprehensive loss has been disclosed in the accompanying statements of operations and comprehensive loss and equals the Company's net loss for all periods presented.

Foreign Currency Translation

The functional currency of the Company's international operations in Canada and Australia is the U.S. dollar. Accordingly, all operating assets and liabilities of these international subsidiaries are remeasured into U.S. dollars using the exchange rates in effect at the balance sheet date or historical rates, as appropriate, while expenses are remeasured into U.S. dollars at the average rates in effect during the period. Any differences resulting from the remeasurement of assets, liabilities, and operations of the Canadian and Australian subsidiaries are recorded within other income (expense), net in the consolidated statements of operations and comprehensive loss. During the years ended December 31, 2016 and 2017, the Company recorded foreign exchange losses of $6 and $19, respectively, in other expense.

Income Taxes

Deferred tax assets and liabilities are determined on the basis of the differences between the consolidated financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which

2. Summary of Significant Accounting Policies (continued)

the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established.

The Company accounts for uncertain tax positions recognized in the consolidated financial statements by prescribing a more-likely-than-not threshold for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Leases

The Company categorizes leases at their inception as either operating or capital leases. On certain lease arrangements, the Company may receive rent holidays or other incentives. The Company recognizes lease costs on a straight-line basis once control of the space is achieved, without regard to deferred payment terms, such as rent holidays, that defer the commencement date of required payments or escalating payment amounts. The difference between required lease payments and rent expense has been recorded as deferred rent and other accrued expenses and other current liabilities in the accompanying consolidated balance sheets. Additionally, incentives received are treated as a reduction of costs over the term of the agreement, as they are considered an inseparable part of the lease agreement.

Net Income (Loss) per Share Attributable to Common Stockholders

Net income (loss) per share attributable to common stockholders is determined using the two-class method, which includes the weighted-average number of shares of common stock outstanding during the period and other securities that participate in dividends (a participating security). In periods of income, the redeemable convertible preferred stock would be considered participating securities because the shares include rights to participate in dividends with the common stock; however, the redeemable convertible preferred stock is not considered a participating security in periods of loss as they do not have an obligation to share in the Company's net losses.

Under the two-class method, basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period. Diluted net income (loss) per share attributable to common stockholders is computed using the more dilutive of (1) the two-class method or (2) the if-converted method. The Company allocates net income first to the holders of Preferred Stock based on dividend rights under the Company's certificate of incorporation and then to preferred and common stockholders based on ownership interests.

Subsequent Event Considerations

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. Subsequent events have been evaluated as required. See Note 17.

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

2. Summary of Significant Accounting Policies (continued)

Emerging Growth Company Status

The Company is an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or JOBS Act, and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. The Company may take advantage of these exemptions until the Company is no longer an "emerging growth company." Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. The Company has elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, its consolidated financial statements may not be comparable to companies that comply with public company effective dates. The Company may take advantage of these exemptions up until the last day of the fiscal year following the fifth anniversary of an offering or such earlier time that it is no longer an "emerging growth company".

Recently Issued Accounting Pronouncements

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) I. Accounting for Certain Financial Instruments with Down Round Features II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception.* Part I applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. For public entities, the amendments in Part I of this update are effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. For all other entities, the amendments in Part I of this update are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted for all entities, including adoption in an interim period. The Company is currently evaluating the impact that the adoption of ASU 2017-11 will have on its consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. The standard is effective for annual periods beginning after December 15, 2017. Early adoption is permitted, including adoption in any interim period, for (1) public business entities for reporting periods for which financial statements have not yet been issued and (2) all other entities for reporting periods for which financial statements have not yet been made available for issuance. The Company is currently evaluating the impact that the adoption of ASU 2017-09 will have on its consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash* ("ASU 2016-18"), which requires restricted cash to be presented with cash and cash equivalents on the statement of cash flows and disclosure of how the statement of cash flows reconciles to the balance sheet if restricted cash is shown separately from cash and cash equivalents on the balance sheet. For public entities, the

2. Summary of Significant Accounting Policies (continued)

standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. For all other entities, the standard is effective for annual periods beginning after December 15, 2018, including interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2016-18 will have on its consolidated financial statements.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments* ("ASU 2016-15"), to address diversity in practice in how certain cash receipts and cash payments are presented and classified in the statement of cash flows. For public entities, the standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. For all other entities, the standard is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. The Company is currently evaluating the impact that the adoption of ASU 2016-15 will have on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less may be accounted for similar to existing guidance for operating leases today. ASU 2016-02 supersedes the previous leases standard, ASC 840, *Leases*. For public entities, not-for-profit entities and an employee benefit plan that files financial statements with the U.S. Securities and Exchange Commission (SEC), the standard is effective for public entities for annual periods beginning after December 15, 2018 including interim periods within those fiscal years. For all other entities, the standard is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted.

In September 2017, the FASB issued ASU No. 2017-13, *Revenue Recognition (Topic 605), Revenue from Contracts with Customers (Topic 606), Leases (Topic 840), and Leases (Topic 842)*, which provides additional clarification and implementation guidance related to ASU 2016-02 and has the same effective date and transition requirements as ASU 2016-02. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its consolidated financial statements.

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

3. Fair Value of Financial Assets and Liabilities

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values as of December 31, 2016 and 2017:

	Fair Value Measurements as of December 31, 2016 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Restricted cash	$ 24	$ —	$ —	$ 24
	$ 24	$ —	$ —	$ 24
Liabilities:				
Derivative liability	$ —	$ —	$ 88	$ 88
	$ —	$ —	$ 88	$ 88

	Fair Value Measurements as of December 31, 2017 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$5,684	$ —	$ —	$5,684
Restricted cash	24	—	—	24
	$5,708	$ —	$ —	$5,708
Liabilities:				
Derivative liability	$ —	$ —	$ 371	$ 371
Warrant to purchase redeemable convertible preferred stock	—	—	35	35
	$ —	$ —	$ 406	$ 406

During the years ended December 31, 2016 and 2017, there were no transfers between Level 1, Level 2 and Level 3.

Valuation of the Warrant to Purchase Preferred Stock

The warrant to purchase redeemable convertible preferred stock liability in the table above is composed of the fair value of a warrant to purchase shares of Series A Preferred Stock that was issued to a lender in connection with a loan and security agreement in 2017 (the "Loan Agreement") (Note 7). The fair value of the warrant to purchase preferred stock was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

In order to determine the fair value of the warrant to purchase preferred stock, the Company utilizes available facts and circumstances to estimate the number of shares of Series A Preferred Stock for which the warrant will ultimately be exercisable. The Company then used the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value the warrant to purchase preferred stock. Estimates and assumptions impacting the fair value measurement include the fair value of the underlying shares of Series A Preferred Stock, the remaining contractual term of the warrant, risk-free interest rate, expected dividend yield and expected volatility of the price of the underlying preferred stock. The Company determined the fair value of the underlying preferred stock based on various valuation methodologies. The Company lacks company-specific

3. Fair Value of Financial Assets and Liabilities (continued)

historical and implied volatility information of its stock. Therefore, it estimates its expected stock volatility based on the historical volatility of publicly traded guideline companies for a term equal to the remaining contractual term of the warrant. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrant. The Company estimated no expected dividend yield based on the fact that the Company has never paid or declared dividends and does not intend to do so in the foreseeable future.

The assumptions that the Company used to determine the fair value of the warrant to purchase preferred stock are as follows:

	June 23, 2017 (Date of issuance)	December 31, 2017
Remaining contractual term (years)	10.00	9.48
Risk-free interest rate	2.15%	2.40%
Expected volatility	80.00%	82.00%
Expected dividend yield	—%	—%
Fair value of Series A Preferred Stock per share	$0.79	$1.42

The following table sets forth a summary of changes in the fair value of the Company's preferred stock warrant liability for which fair value is determined by Level 3 inputs:

	Warrant Liability
Balance as of December 31, 2016	$ —
Initial fair value of warrant to purchase redeemable convertible preferred stock	18
Change in fair value	17
Balance as of December 31, 2017	$ 35

Valuation of Derivative

In January 2016, in connection with a license agreement entered into with University Health Network ("UHN"), and as part of the initial consideration for the license, the Company issued 1,161,665 shares of common stock to UHN pursuant to a stock purchase agreement (the "Stock Purchase Agreement"). See Note 11 for further discussion on the license agreement. The shares were fully vested on the date of issuance and did not contain any restrictions. The Stock Purchase Agreement contains a provision requiring the Company to make a cash payment to UHN of up to $2,000 if UHN's fully diluted ownership is reduced within specified percentages as part of an IPO by the Company. The Company concluded the anti-dilution feature represented a derivative instrument and should be measured at fair value, with changes in fair value recognized as a gain or loss to other income (expense), net in the consolidated statements of operations and comprehensive loss. The initial fair value of the derivative of $49 was recorded as research and development expense in January 2016.

On December 31, 2016 and 2017, the Company remeasured the fair value of the derivative, using current assumptions, resulting in an increase in fair value of $39 and $283, respectively, which was recorded in other expense in the accompanying consolidated statements of operations and comprehensive loss for the years ended December 31, 2016 and 2017. The Company will continue to re-measure the fair value of the liability at the end of each reporting period until the completion of an IPO.

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

3. Fair Value of Financial Assets and Liabilities (continued)

The following table sets forth a summary of changes in the fair value of the Company's derivative liability for which fair value is determined by Level 3 inputs:

	Derivative Liability
Balance as of December 31, 2015	$ —
Initial fair value of derivative liability	49
Change in fair value	39
Balance as of December 31, 2016	88
Change in fair value	283
Balance as of December 31, 2017	$ 371

4. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31,	
	2016	2017
Prepaid research and development costs	$ —	$ 163
Prepaid rent	15	122
Other current assets	4	60
	$ 19	$ 345

5. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31, 2017
Laboratory and office equipment	$ 126
Leasehold improvements	240
Computer equipment and software	28
	394
Less: Accumulated depreciation and amortization	(45)
	$ 349

As of December 31, 2016, the Company did not hold any property and equipment. Depreciation and amortization expense for the year ended December 31, 2017 was $45.

6. Accrued Expenses

Accrued expenses consisted of the following:

	December 31,	
	2016	2017
Compensation and benefit costs	$374	$ 794
Research and development costs	—	831
Consulting and professional fees	89	267
Preferred stock issuance cost	—	85
Other liabilities	88	121
	$551	$2,098

7. Loan Agreement and Warrant to Purchase Preferred Stock

On June 23, 2017, the Company entered into the Loan Agreement with a lender, which provided for the issuance of term loans of up to $10,000, subject to the achievement of various development and corporate milestones. Any outstanding principal amounts under the Loan Agreement will accrue interest at a floating per annum rate equal to the greater of 1% and the “prime rate,” as defined in the Loan Agreement, minus 3%. Payments on the Loan Agreement are interest only, payable monthly in arrears, until November 1, 2018, which can be extended by six months if the third tranche is drawn. Thereafter, principal and interest amounts are repayable over a 30-month period, unless the third tranche is funded and the initial interest-only period is extended by six months, in which case principal and interest amounts are repayable over a 24-month period. As of December 31, 2017, the Company had not drawn down from the facility and $3,500 was available to the Company.

The Loan Agreement contains customary indemnification obligations and customary events of default. In the event of default by the Company under the Loan Agreement, the lender would be entitled to exercise their remedies thereunder, including the right to accelerate the debt, upon which the Company may be required to repay all amounts then outstanding under the Loan Agreement or the lender may take possession of the collateral securing the Loan Agreement. No events of default had occurred through December 31, 2017.

The Loan Agreement also includes certain restrictions on, among other things, the Company’s ability to incur additional indebtedness, change the name or location of its business, merge with or acquire other entities, pay dividends or make other distributions to holders of the Company’s capital stock, make certain investments, engage in transactions with affiliates, create liens, open new deposit accounts, sell assets or pay subordinated debt.

In connection with the Loan Agreement, the Company agreed to issue a warrant to the lender for the purchase of up to 188,702 shares of the Company’s Series A Preferred Stock with an exercise price of $0.7949 per share. The warrant expires on June 22, 2027. The warrant is initially exercisable for the purchase of up to 28,305 shares of Series A Preferred Stock and can become exercisable for up to an additional 160,397 shares of Series A Preferred Stock based on the amounts drawn under the Loan Agreement. On the issuance date of the warrant, the Company recorded a deferred financing cost and a liability for the warrant to purchase preferred stock in the Company’s consolidated balance sheet equal to the issuance-date fair value of the warrant. As of December 31, 2017, the warrant is exercisable for up to 28,305 shares of Series A Preferred Stock.

7. Loan Agreement and Warrant to Purchase Preferred Stock (continued)

On the date of issuance, the fair value of the warrant was determined to be $18. The Company remeasured the liability associated with the warrant as of December 31, 2017 and determined that the fair value of the preferred stock warrant liability was $35. The Company recognized a loss of $17 in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2017, related to the change in fair value of the warrant.

8. Redeemable Convertible Preferred Stock

As of December 31, 2016, the authorized capital stock of the Company included 22,380,952 shares of $0.0001 par value preferred stock, of which 3,333,333 shares have been designated as Series Seed Preferred Stock and 19,047,619 shares have been designated as Series A Preferred Stock. As of December 31, 2017, the authorized capital stock of the Company included 34,972,535 shares of $0.0001 par value preferred stock, of which 3,333,333 shares have been designated as Series Seed Preferred Stock and 31,639,202 shares have been designated as Series A Preferred Stock.

In January 2016, the Company issued and sold 3,333,333 shares of Series Seed Preferred Stock at a price of $0.45 per share, for total proceeds of $1,398, net of issuance costs of $102.

In July 2016, the Company issued and sold 5,714,286 shares of Series A Preferred Stock, at a price of $1.3125 per share, for total proceeds of $7,297, net of issuance costs of $203. The terms of the Series A Preferred Stock Purchase Agreement included the obligation of the investors to purchase, and the Company to sell, up to 13,333,333 additional shares of Series A Preferred Stock at $1.3125 per share contingent upon the achievement of certain specified milestones or at the election of a required vote by the investors and the Board. The Company concluded that the right to participate in the future issuance of Series A Preferred Stock did not meet the definition of a freestanding financial instrument, as the rights were not legally detachable from the Series A Preferred Stock.

In March 2017, the Company amended certain provisions of the Series A Preferred Stock. The changes included issuing additional instruments through the preferred stock dividend in the amount of 3,720,864 shares of Series A Preferred Stock, which effectively repriced the outstanding shares of Series A Preferred Stock, changing the purchase price for future shares, and decreasing the redemption price per share such that total redemption value was not affected. Concurrent with the amendment, the Company issued 4,403,070 additional shares of Series A Preferred Stock at a purchase price of $0.7949 per share, for total proceeds of $3,452, net of issuance costs of $48. Additionally, the number of shares of Series A Preferred Stock which could be issued in the future was changed to 17,612,279 shares. The Company assessed the changes to the terms and concluded that it represented a material change to substantive terms of the instrument, and therefore represented, for accounting purposes, an extinguishment and re-issuance of the Series A Preferred Stock outstanding at the time. As the carrying value of the outstanding shares of Series A Preferred Stock prior to the extinguishment was deemed to equal the aggregate fair value of the reissued shares and the shares issued as a dividend, no gain or loss was recognized on extinguishment of the Series A Preferred Stock.

In October 2017, the Company issued and sold an additional 17,612,279 shares of Series A Preferred Stock, at a price of $0.7949 per share, for total proceeds of $13,963, net of issuance costs of $37.

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

8. Redeemable Convertible Preferred Stock (continued)

As of each balance sheet date, the Preferred Stock consisted of the following:

	December 31, 2016				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series Seed preferred stock	3,333,333	3,333,333	$1,500,000	$1,500,000	806,711
Series A preferred stock	19,047,619	5,714,286	7,500,000	7,500,000	1,382,933
	22,380,952	9,047,619	$9,000,000	$9,000,000	2,189,644

	December 31, 2017				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series Seed preferred stock	3,333,333	3,333,333	$ 1,500,000	$ 1,500,000	806,711
Series A preferred stock	31,639,202	31,450,499	25,000,000	25,000,000	7,611,438
	34,972,535	34,783,832	$ 26,500,000	$ 26,500,000	8,418,149

As of December 31, 2017, the holders of the Preferred Stock have the following rights and preferences:

Voting Rights—

The holders of the Preferred Stock are entitled to vote, together with the holders of common stock, on all matters submitted to the stockholders for a vote and are entitled to the number of votes equal to the number of whole shares of common stock into which such holders of Preferred Stock could convert on the record date for determination of stockholders entitled to vote. Except for the actions requiring the approval or consent of the majority of the holders of Preferred Stock, the holders of Preferred Stock shall vote together with the holders of common stock and vote as a single class.

Dividends—

The holders of the Preferred Stock are entitled to receive noncumulative dividends when, as and if declared by the Board. The Company may not pay any dividends on shares of common stock of the Company unless the holders of Preferred Stock then outstanding simultaneously receive dividends at least equal to a fixed percentage of Preferred Stock. Through December 31, 2017, no cash dividends have been declared or paid.

Liquidation Rights—

In the event of any voluntary or involuntary liquidation event, dissolution, winding up of the Company or upon the occurrence of certain events designated by a majority of the holders of the Preferred Stock to be a deemed liquidation event, each holder of the then outstanding Series A Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of Series Seed Preferred Stock and common stock, an amount equal to the greater of (i) $0.7949 per share (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

8. Redeemable Convertible Preferred Stock (continued)

After the payment of all preferential amounts to the holders of Series A Preferred Stock, each holder of the then outstanding Series Seed Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of common stock, an amount equal to the greater of (i) $0.45 per share (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

After payments have been made in full to the holders of the Preferred Stock, then, to the extent available, the remaining amounts will be distributed among the holders of the shares of common stock, pro rata based on the number of shares held by each holder.

Conversion—

Each share of Preferred Stock is convertible into common stock, at any time, at the option of the holder, and without the payment of additional consideration, at the applicable conversion ratio then in effect for each series of Preferred Stock, initially set to be one-for-one, and subject to adjustment in accordance with anti-dilution provisions. In addition, each share of Preferred Stock will be automatically converted into common stock at the applicable conversion ratio then in effect for each series of Preferred Stock upon the earlier of a qualified IPO which results in net proceeds of at least $50,000 and the listing of the Company's common stock on the New York Stock Exchange or the Nasdaq Stock Market, or upon a vote of the holders of a majority of the outstanding Preferred Stock. As of December 31, 2017, each share of Preferred Stock was convertible into 0.242 shares of common stock and can be adjusted in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock.

The Company evaluated each series of its Preferred Stock and determined that each individual series is considered an equity host. In making this determination, the Company's analysis followed the whole instrument approach which compares an individual feature against the entire preferred stock instrument which includes that feature. The Company's analysis was based on a consideration of the economic characteristics and risks of each series of Preferred Stock. More specifically, the Company evaluated all of the stated and implied substantive terms and features, including: (1) whether the Preferred Stock included redemption features, (2) how and when any redemption features could be exercised, (3) whether the holders of Preferred Stock were entitled to dividends, (4) the voting rights of the Preferred Stock and (5) the existence and nature of any conversion rights. As a result of the Company's conclusion that the Preferred Stock represents an equity host, the conversion feature of all series of Preferred Stock is considered to be clearly and closely related to the associated Preferred Stock host instrument. Accordingly, the conversion feature of all series of Preferred Stock is not considered an embedded derivative that requires bifurcation.

The Company accounts for potential beneficial conversion features at the time of issuance. The Company's common stock into which each series of the Company's Preferred Stock is convertible had an estimated fair value less than the effective conversion prices of the Preferred Stock at the time of each of the issuances of Preferred Stock. Therefore, there was no intrinsic value on the respective commitment dates. In addition, the Company considered the other features included within the Preferred Stock and determined that none of the other features required bifurcation and separate accounting.

8. Redeemable Convertible Preferred Stock (continued)

Redemption—

The Series Seed Preferred Stock and Series A Preferred Stock are redeemable at $0.45 per share and $0.7949 per share, respectively, (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends, on or after July 21, 2021 at the written election of at least a majority of the holders of Preferred Stock voting together as a single class. The redemption is paid in three annual installments. No bifurcation of the redemption feature was required as the feature does not contain the characteristics of a derivative instrument.

As the Preferred Stock is redeemable upon the passage of time, the Preferred Stock has been classified outside of permanent equity. The Company has elected to record the changes in the redemption value immediately as they occur.

9. Common Stock

As of December 31, 2016 and 2017, the authorized capital stock of the Company included 40,000,000 and 51,000,000 shares of common stock, $0.0001 par value, respectively. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth above.

On January 27, 2016, the Company modified the terms of 330,750 shares of common stock which were issued to four employees in November 17, 2015 to add a vesting condition. The restrictions lapse according to the time-based vesting conditions of each award. During each of the years ended December 31, 2016 and 2017, 82,686 shares of these restricted common stock awards vested.

Each share of common stock entitles the holder to one vote, together with the holders of the Preferred Stock, on all matters submitted to the stockholders for a vote. The holders of the Preferred Stock, voting as a single class, are entitled to elect three directors of the Company. The holders of common stock, together with the holders of the Preferred Stock and voting as a single class, are entitled to elect two directors of the Company. The holders of common stock and of any other class or series of voting stock (including the Preferred Stock), voting together as a single class, are entitled to elect the remaining directors of the Company. Common stockholders are entitled to receive dividends, as may be declared by the Board, if any, subject to the preferential dividend rights of the Preferred Stock. Through December 31, 2017, no cash dividends have been declared or paid.

At December 31, 2016 and 2017, the Company has reserved the following shares of common stock for future issuance:

	December 31,	
	2016	2017
Shares reserved for Series Seed Preferred Stock outstanding	806,711	806,711
Shares reserved for Series A Preferred Stock outstanding	1,382,933	7,611,438
Shares reserved for vesting of restricted stock awards	409,406	276,301
Shares reserved for exercise of outstanding stock options	599,439	1,034,961
Shares reserved for issuance under the 2015 Stock Option and Grant Plan	52,710	143,717
Total shares of authorized common stock reserved for future issuance	3,251,199	9,873,128

10. Stock-Based Compensation

Amended and Restated 2015 Stock Option and Grant Plan

The Company's Amended and Restated 2015 Stock Option and Grant Plan, (the "2015 Plan") provides for the Company to issue restricted stock awards and restricted stock units, or to grant incentive stock options or non-statutory stock options. Incentive stock options may be granted only to the Company's employees including officers and members of the Board who are also employees. Restricted stock awards and restricted stock units and non-statutory stock options may be granted to employees, members of the Board, outside advisors, and consultants of the Company.

The total number of common shares that may be issued under the 2015 Plan was 1,340,020 shares as of December 31, 2017, of which 143,717 shares remained available for future grant.

Shares that expire, are terminated, surrendered or canceled under the 2015 Plan without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future awards.

The 2015 Plan is administered by the Board. Equity awards granted to employees and members of the Board typically vest over four years.

During the years ended December 31, 2016 and 2017, the Company granted options to purchase 599,439 shares and 433,102 shares, respectively, of common stock to employees and members of the Board.

During the year ended December 31, 2017, the Company granted options to purchase 2,420 shares of common stock to a non-employee. The stock-based compensation expense for options granted to non-employees is nominal during the year ended December 31, 2017. The Company did not grant options to purchase shares of common stock to non-employees during the year ended December 31, 2016.

Stock Option Valuation

The assumptions that the Company used to determine the grant-date fair value of stock options granted to employees and members of the Board were as follows, presented on a weighted-average basis:

	Year Ended December 31,	
	2016	2017
Expected option life (years)	6.00	6.08
Risk-free interest rate	1.39%	1.93%
Expected volatility	86.00%	84.54%
Expected dividend yield	—%	—%

AVROBIO, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

10. Stock-Based Compensation (continued)

The following table summarizes the Company's stock option activity for the year ended December 31, 2017:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2016	599,439	$ 0.69	9.47	$ 307
Granted	435,522	$ 0.91		
Exercised	—	$ —		
Cancelled or forfeited	—	$ —		
Outstanding as of December 31, 2017	1,034,961	$ 0.78	8.94	$ 3,427
Exercisable as of December 31, 2017	185,446	$ 0.68	8.46	$ 633
Unvested as of December 31, 2017	849,515	$ 0.81	9.04	$ 2,794

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the underlying stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the estimated fair value of the Company's common stock at December 31, 2017.

The weighted-average grant-date fair value of the Company's stock options granted during the years ended December 31, 2016 and 2017 was $0.50 and $1.50, respectively.

Restricted Common Stock

The Company has granted restricted common stock with time-based vesting conditions to certain employees of the Company. The purchase price of the restricted stock awards are determined by the Board. The Company also, in January 2016, modified 330,750 shares of common stock which were issued to four employees in November 17, 2015 to add a vesting condition (see Note 9). Unvested shares of restricted common stock may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. The Company has the option to repurchase the restricted stock awards at the original purchase price if the grantee terminates its working relationship with the Company prior to the vesting date.

The following table summarizes the Company's restricted common stock activity for the year ended December 31, 2017:

	Number of Shares	Weighted-Average Grant Date Fair Value	Aggregate Intrinsic value
Issued and unvested as of December 31, 2016	409,406	$ 0.42	$ 1,675
Vested	(133,105)	$ 0.42	$ 544
Forfeited, canceled or expired	—	$ —	$ —
Issued and unvested as of December 31, 2017	276,301	$ 0.42	$ 1,130

10. Stock-Based Compensation (continued)

The weighted-average grant date fair value of restricted common stock awards granted during the year ended December 31, 2016 was $0.42 per share. The total fair value of restricted common stock vested during the years ended December 31, 2016 and 2017 was $34 and $55, respectively.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows:

	Year Ended December 31,	
	2016	2017
Research and development	$ 32	$ 80
General and administrative	40	97
Total stock based compensation expense	$ 72	$ 177

As of December 31, 2017, total unrecognized compensation cost related to the unvested stock-based awards was $897, which is expected to be recognized over a weighted-average period of 3.26 years.

11. License Agreements

Agreements with UHN

Fabry License Agreement—

On January 27, 2016, the Company entered into an agreement with UHN, pursuant to which UHN granted the Company an option to enter into an exclusive license under the UHN intellectual property related to Fabry disease in accordance with the pre-negotiated licensing terms. On November 4, 2016, the Company exercised its option and entered into a license agreement with UHN, pursuant to which UHN granted the Company an exclusive worldwide license under certain intellectual property rights and a non-exclusive worldwide license under certain know-how, in each case subject to certain retained rights, to develop, commercialize and sell products for use in the treatment of Fabry disease. In addition, for three years following the execution of the agreement, UHN granted the Company an exclusive option to obtain a license under certain improvements to the licensed intellectual property rights as well as an option to negotiate a license under certain other improvements.

Under this agreement, the Company paid an option fee of CAD $20, an upfront license fee of CAD $75, plus the annual license maintenance fee for the first year. Thereafter, the Company is also required to pay UHN future annual license maintenance fees until the first sale of a licensed product in certain markets. The Company is also obligated to make future milestone payments in an aggregate amount of up to CAD $2,450 upon the achievement of specified milestones as well as royalties on a country-by-country basis of a low to mid-single-digit percentage of annual net sales of licensed products and a lower single-digit royalty percentage in certain circumstances. Additionally, the Company has agreed to pay a low double-digit royalty percentage of all sublicensing revenue.

The agreement requires the Company to meet certain performance milestones within specified timeframes. UHN may terminate the agreement if the Company fails to meet these performance milestones despite using commercially reasonable efforts and the Company is unable to reach agreement with UHN on revised

11. License Agreements (continued)

timeframes. The Company's royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the latest to occur of the expiration or termination of the last valid claim under the licensed intellectual property rights in such country, the tenth anniversary of the first commercial sale of such licensed product in such country and the expiration of any applicable regulatory exclusivity in such country.

Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products. UHN can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event that the Company fails to obtain or maintain insurance. Either the Company or UHN may terminate the license agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company can voluntarily terminate the agreement with prior notice to UHN.

For the years ended December 31, 2016 and 2017, the Company recorded research and development expense of $87 and $16, respectively, which consists of the license option fee, the upfront fee and maintenance fees.

Interleukin 12 License Agreement—

On January 27, 2016, the Company entered into an exclusive license agreement with UHN, pursuant to which UHN granted the Company a license to certain patent rights for the commercial development, manufacture, distribution and use of any products or processes resulting from development of those patent rights related to Interleukin 12. Upon execution of this agreement, the Company paid an upfront license fee of CAD $264. In addition, as part of the initial consideration for the license, the Company issued to UHN 1,161,665 shares of the Company's common stock. The fair value of the shares issued to UHN of $480 and the upfront fee was expensed upon the execution of the agreement. In addition, the Company agreed to pay UHN up to $2,000 upon the closing of an IPO if certain criteria are met. This obligation is considered a derivative instrument and was initially recorded at fair value of $49. The Company is also required to pay UHN future annual license maintenance fees of CAD $50 on each anniversary of the effective date of the license agreement prior to expiration or termination and potential future milestone payments of up to CAD $19,275 upon the achievement of specified clinical and regulatory milestones. The Company also agreed to pay UHN royalties of a low single-digit percentage of net sales of licensed products sold by the Company. If the Company grants any sublicense rights under the license agreement, the Company has agreed to pay UHN a low double-digit royalty percentage of any sublicense income received by the Company.

The agreement requires the Company to meet certain due diligence requirements based upon specified milestones. The agreement expires on the later of the date the last patent rights expire in the last country or ten years from the date of first sale. UHN can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event that the Company fails to obtain or maintain insurance. The Company can voluntarily terminate the agreement with prior notice to UHN. Either the Company or UHN may terminate the license agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time.

For the years ended December 31, 2016 and 2017, the Company recorded research and development expense related to this agreement with UHN of $785 and $151, respectively, which consists of upfront fees, the fair value of the shares and derivative instrument issued to UHN, and license maintenance fees and development milestone payments.

11. License Agreements (continued)

Agreement with BioMarin Pharmaceutical Inc. ("BioMarin")

On August 31, 2017, the Company entered into a license agreement with BioMarin, pursuant to which BioMarin granted the Company an exclusive worldwide license under certain intellectual property rights owned or controlled by BioMarin to develop, commercialize and sell products for use in the treatment of Pompe disease. As consideration for this agreement, the Company paid an upfront license fee of $500 in cash and issued 233,765 shares of Series B Preferred Stock to BioMarin at the time of our Series B Preferred Stock financing in January 2018. Both the upfront cash payment of $500 and the value of the shares Series B Preferred Stock issued of $500 were recorded as research and development expense during the year ended December 31, 2017. The Company is also obligated to make future milestone payments of up to $13,000 upon the achievement of certain specified milestones and agreed to pay BioMarin royalties of a low single-digit percentage of net sales of licensed products sold by the Company or its affiliates covered by patent rights in a relevant country.

Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products throughout the world. BioMarin and the Company can terminate the agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company may terminate the agreement at will upon written notice to BioMarin. BioMarin has the right to terminate the agreement upon the Company's bankruptcy or insolvency, or in the event of any challenge or opposition to the licensed patent rights or related actions brought by the Company or its affiliates or sublicensees, or if the Company, its affiliates or sublicensees knowingly assist a third-party in challenging or otherwise opposing the licensed patent rights, except as required under a court order or subpoena.

Agreement with GenStem Therapeutics, Inc. ("GenStem")

On October 2, 2017, the Company entered into a license agreement with GenStem, pursuant to which GenStem granted the Company an exclusive worldwide license, subject to certain retained rights, under certain intellectual property rights owned or controlled by GenStem to develop, commercialize and sell products for use in the treatment of cystinosis. Under this agreement, the Company paid an upfront license fee of $1,000 and is required to make payments upon completion of certain milestones up to an aggregate of $16,000. The Company also agreed to pay GenStem a tiered mid to high single-digit royalty percentage on annual net sales of licensed products as well as a low double-digit percentage of sublicense income received from certain third party licensees. The Company's royalty obligation expires on a licensed product-by-licensed product and country-by-country basis on the eleventh anniversary of the first commercial sale of such licensed product in such country or the expiration of the last valid claim under the licensed patent rights covering such licensed product in such country, whichever is later. Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products throughout the world. GenStem and the Company can terminate the agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company may terminate the agreement at will upon the specified prior written notice to GenStem. The Company recorded research and development expense of $1,000 for the year ended December 31, 2017, which consisted of upfront fees related to the license.

Agreement with Lund University Rights Holders

On November 17, 2016, the Company entered into a license agreement with affiliates of Lund University, along with certain other relevant rights holders that may be added from time to time, pursuant to which such rights holders granted to the Company an exclusive worldwide license, subject to certain retained rights, under

11. License Agreements (continued)

certain intellectual property rights to develop, commercialize and sell products in any and all uses relevant to Gaucher disease. As consideration for the license, the Company is required to make payments in connection with the achievement of certain milestones up to an aggregate of $550. The agreement expires on the latest of (i) the twentieth anniversary of the end of a certain research project the Company is funding pursuant to an agreement with Lund University, (ii) the expiration of the term of any patent filed on the licensed rights that covers a licensed product, (iii) the expiration of any applicable marketing exclusivity right and (iv) such time that neither the Company nor any sublicensees, partners or contractors are commercializing a licensed product. Either the Company or the rights holders acting together may terminate the license agreement if the other such party commits a material breach and fails to cure such breach within a certain period of time, or if the other party enters into liquidation, becomes insolvent, or enters into composition or statutory reorganization proceedings.

12. Income Taxes

For the years ended December 31, 2016 and 2017, the Company did not record a current or deferred income tax expense or (benefit) due to current and historical losses incurred by the Company. The Company's operations are predominantly based in the United States and the Company's foreign subsidiaries generated de minimis profit for the years ended December 31, 2016 and 2017.

The enactment of the Tax Cuts and Jobs Act ("TCJA") in December 2017, as further described below, resulted in a remeasurement of the Company's net deferred tax asset due to the reduction in the corporate income tax rate from 35% to a 21% flat tax, which is included in the Company's 2017 rate reconciliation.

A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate to income taxes as reflected in the consolidated financial statements is as follows:

	Year Ended December 31,	
	2016	2017
Federal income tax expense at statutory rate	34.0%	34.0%
State income taxes, net of federal benefit	5.1	4.9
Permanent differences	(0.8)	(0.3)
U.S.—TCJA	—	(14.8)
Foreign rate differential	—	(0.2)
Research and development tax credits	2.3	1.3
Change in valuation allowance	(40.6)	(24.9)
Effective income tax rate	—%	—%

12. Income Taxes (continued)

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The significant components of the Company's deferred tax assets and liabilities are comprised of the following:

	December 31, 2016	December 31, 2017
Deferred tax assets:		
U.S., foreign, and state net operating loss carryforwards	$ 1,435	$ 5,183
Research and development credits	19	95
Capitalized start up and organizational costs	60	39
Equity based compensation	18	29
Derivative liability	35	101
Licensing agreements	331	800
Accruals and other	—	239
Total deferred tax assets	1,898	6,486
Valuation allowance	(1,898)	(6,466)
Net deferred tax assets	$ —	$ 20
Deferred tax liabilities:		
Property and equipment	$ —	$ (20)
Total deferred tax liabilities	$ —	$ (20)

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2016 and 2017, based on the Company's history of operating losses, the Company has concluded that it is not more likely than not that the benefit of its deferred tax assets will be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of December 31, 2016 and 2017. The valuation allowance increased $1,898 and $4,568 during the years ended December 31, 2016 and 2017, respectively, due primarily to net operating losses generated.

As of December 31, 2016 and 2017, the Company had U.S. federal net operating loss carryforwards of $3,659 and $19,007, respectively, that may be available to offset future income tax liabilities. The TCJA will generally allow losses incurred after 2017 to be carried over indefinitely, but will generally limit the net operating loss deduction to the lesser of the net operating loss carryover or 80% of a corporation's taxable income. Also, there will be no carryback for net operating losses incurred after 2017. Net operating losses incurred prior to 2018 will generally be deductible to the extent of the lesser of a corporation's net operating loss carryover or 100% of a corporation's taxable income, and be available to offset future taxable income for a period of twenty years.

The Company has early adopted the provisions of ASU 2016-09, *Compensation—Stock Compensation (Topic 718 Improvements to Employee Share-Based Payment Accounting)*, for its year ended December 31, 2016. ASU 2016-09 requires companies to include the benefit of an option deduction in its net operating loss carryforward deferred tax asset. The Company did not have any deductions associated with stock-based payments, and therefore the adoption of ASU 2016-09 did not impact the Company's deferred tax asset for net operating loss carryforwards. Furthermore, since the Company has historically maintained a full valuation allowance on its net worldwide deferred tax asset, there is no net impact to retained earnings from the adoption of ASU 2016-09.

12. Income Taxes (continued)

As of December 31, 2016 and 2017, the Company also had U.S. state net operating loss carryforwards of $3,619 and $18,866, respectively, which may be available to offset future taxable income. These losses expire at various dates through 2037.

As of December 31, 2016 and 2017, the Company does not have federal research and development tax credit carryforwards, as the Company qualifies for, and has elected to, apply such federal research credits against its payroll tax liability in accordance with certain provisions of the Internal Revenue Code. As of December 31, 2016 and 2017, the Company had state research and development tax credit carryforwards of approximately $30 and $119, respectively, available to reduce future tax liabilities which expire at various dates through 2032. For all years through December 31, 2017, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards.

Under the provisions of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed numerous financings since its inception, which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company files income tax returns in Australia, Canada, the United States, and in several states. The foreign, federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2015 through December 31, 2017. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by foreign tax authorities, the Internal Revenue Service, or state tax authorities to the extent utilized in a future period.

The TCJA was enacted in December 2017. Among other things, the TCJA reduces the U.S. federal corporate tax rate from 35% to 21% for tax years beginning in 2018, and requires companies to pay a one-time transition tax on previously unremitted earnings of non-U.S. subsidiaries that were previously tax deferred and creates new taxes on certain foreign sourced earnings. The SEC staff issued Staff Accounting Bulletin ("SAB") 118, that provides guidance on accounting for enactment effects of the TCJA. SAB 118 provides a measurement period of up to one year from the TCJA's enactment date for companies to complete their accounting under ASC 740. In accordance with SAB 118, to the extent that a company's accounting for certain income tax effects of the TCJA is incomplete but it is able to determine a reasonable estimate, it must record a provisional estimate in its consolidated financial statements. If a company cannot determine a provisional estimate to be included in its consolidated financial statements, it should continue to apply ASC 740 on the basis of the provisions of the tax laws that were in effect immediately before the enactment of the TCJA.

In connection with the Company's initial analysis of the impact of the enactment of the TCJA, the Company has not recorded an income tax expense. For various reasons that are discussed more fully below, the Company has not completed its accounting for the income tax effects of certain elements of the TCJA. However, with

12. Income Taxes (continued)

respect to the remeasurement of deferred tax assets and liabilities, the Company has remeasured its deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21% under the TCJA. The impact of the remeasurement of the Company's deferred tax assets and liabilities is included in the rate reconciliation above.

The Company is still analyzing certain aspects of the TCJA, including considering additional technical guidance and refining its calculations, that could potentially affect the measurement of these balances or potentially give rise to new deferred tax amounts. This includes the potential impacts of the global low-taxed income ("GILTI") provision within the TCJA on deferred tax assets and liabilities. The Company has not yet elected a policy as to whether it will recognize deferred taxes for basis differences expected to reverse as GILTI or whether the Company will account for GILTI as a period cost if and when incurred. Additionally, with respect to the transition tax, which is a tax on previously untaxed accumulated and current earnings and profits (E&P) of certain of the Company's non-U.S. subsidiaries. The Company is currently evaluating the impact of this issue, and has not finalized a conclusion at this time and therefore cannot determine a provisional estimate to be included in its consolidated financial statements, and in accordance with SAB 118 it will continue to apply ASC 740 on the basis of the provisions of the tax laws that were in effect immediately before the enactment of the TCJA.

13. Net Loss per Share and Unaudited Pro Forma Net Loss per Share

Net Loss per Share Attributable to Common Stockholders

For purposes of the diluted net loss per share calculation, stock options, unvested restricted stock, Preferred Stock and the warrant to purchase shares of Series A Preferred Stock are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, the weighted-average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same.

The following potentially dilutive common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Year Ended December 31,	
	2016	2017
Options to purchase common stock	599,439	1,034,961
Restricted common stock	409,406	276,301
Redeemable convertible preferred stock (as converted to common stock)	2,189,644	8,418,149
Warrants to purchase redeemable convertible preferred stock (as converted to common stock)	—	6,850

Unaudited Pro Forma Net Loss per Share Attributable to Common Stockholders

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2017 has been prepared to give effect to adjustments arising upon the closing of a qualified IPO. The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders does not include

13. Net Loss per Share and Unaudited Pro Forma Net Loss per Share (continued)

the effects of the accretion of Preferred Stock to redemption value or the change in fair value of the warrant to purchase shares of Series A Preferred Stock because the calculation gives effect to the conversion of shares of Preferred Stock outstanding as of December 31, 2017 into common stock and the conversion of the warrant to purchase shares of Series A Preferred Stock outstanding as of December 31, 2017 into a warrant to purchase shares of common stock, as if such conversion had occurred at the beginning of the period presented or the date of original issuance, whichever is later.

A reconciliation of pro forma net loss and the pro forma weighted-average number of common shares used in computing pro forma basic and diluted net loss per share applicable to common stockholders is as follows:

	Year Ended December 31, 2017 (unaudited)
Numerator:	
Net loss attributable to common stockholders	$ (18,733)
Accretion of issuance costs on redeemable convertible preferred stock	85
Change in the fair value of preferred stock warrant liability	17
Pro forma net loss attributable to common stockholders	$ (18,631)
Denominator:	
Weighted-average number of common shares used in computing net loss per share attributable to common stockholders—basic and diluted	2,235,865
Pro forma adjustment to reflect assumed conversion of redeemable convertible preferred stock into common stock	4,686,308
Pro forma weighted-average number of common shares used in computing pro forma net loss per share attributable to common stockholders—basic and diluted	6,922,173
Pro forma net loss per share attributable to common stockholders—basic and diluted	$ (2.69)

14. Commitments and Contingencies

Lease Agreements

In January 2016, the Company entered into a sub-lease agreement for office space located in Cambridge Massachusetts, United States, which was a month to month rental. In February 2017, the Company terminated the office lease agreement.

In September 2016, the Company entered into a lease agreement for office space located in Cambridge Massachusetts, United States, which expires on March 31, 2024. The base rent will be increased by 3% annually. The Company recognizes rent expense on a straight-line basis over the lease period and has recorded deferred rent for rent expense incurred but not yet paid. The Company received a tenant incentive allowance of $100 in 2017. Such incentive allowance is being amortized as a reduction of rent expense on a straight-line basis over the lease period. In accordance with the lease agreement, the Company was required to maintain a security deposit of $24. The Company issued a letter of credit to the landlord related to the security deposit and the letter of credit is secured by restricted cash, which is recorded in other assets on the accompanying consolidated balance sheets.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

14. Commitments and Contingencies (continued)

In August 2017, the Company entered into a sub-lease agreement for laboratory space located in Cambridge Massachusetts, United States, which expires in August 2020. The annual lease payments are subject to a 3% increase each year. The Company recognizes rent expense on a straight-line basis over the lease period and has recorded deferred rent for rent expense incurred but not yet paid.

The Company recorded rent expense of $78 and $335 during the years ended December 31, 2016 and 2017, respectively.

The following table summarizes the future minimum lease payments due under operating leases as of December 31, 2017:

Year Ending December 31,	
2018	$ 395
2019	380
2020	302
2021	168
2022	173
Thereafter	223
	$1,641

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the years ended December 31, 2016 and 2017, and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

Other

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at December 31, 2016 and 2017, or royalties on future sales of specified products. No milestone or royalty payments under these agreements are expected to be payable in the immediate future. See Note 11 for discussion of these arrangements.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company agrees to indemnify, hold harmless, and to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third-party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

15. Related Party Transactions

UHN

In connection with the Company's entry into a license agreement with UHN (Note 11) on January 27, 2016, the Company issued UHN 4,800,000 shares of its common stock. As a result of the issuance of common stock,

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Years ended December 31, 2016 and 2017

15. Related Party Transactions (continued)

UHN owned 21.63% and 9.65% of the Company's fully diluted equity as of December 31, 2016 and 2017, respectively. Upon the closing of the sale of shares of common stock in an IPO, if UHN's fully-diluted percentage ownership of the Company is reduced within a range of specified percentages, then the Company is obligated to pay UHN an amount up to $2,000. See Note 3 for further discussion on the accounting treatment for this provision.

During the years ended December 31, 2016 and 2017, the Company recognized $872 and $167 respectively, of research and development expense related to the license agreements with UHN. Refer to Note 11 for additional information regarding the UHN license agreements.

The Company recorded research and development expenses of $7 and $3 related to participation on the scientific advisory board and consulting services performed by a member of the Board who is affiliated with UHN during the years ended December 31, 2016 and 2017, respectively. As of December 31, 2016 and 2017, there was $7 and $10, respectively, included in accrued expenses on the Company's consolidated balance sheets related to these services.

For the years ended December 31, 2016 and 2017 the Company recorded expenses of $13 and $86, respectively, related to consulting services provided by an entity affiliated with an officer of the Company and a member of the Board. The entity is also a shareholder of the Company and owned 2.25% and 1.00% of the Company's fully diluted equity as of December 31, 2016 and 2017, respectively.

Others

For the years ended December 31, 2016 and 2017, the Company recorded expenses of $78 and $15, respectively, related to services provided by an entity affiliated with a member of the Board and the use of office space.

In August 2017, the Company entered into a sub-lease agreement with an entity affiliated with the Company's CEO. The executive resigned from the affiliated entity in September 2017. The Company recorded rent expense of $31 under this sub-lease agreement prior to such resignation for the two month period in 2017.

See Note 14 for additional information on the terms of these sublease agreements.

16. Benefit Plans

The Company established a defined contribution savings plan under Section 401(k) of the Internal Revenue Code. This plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Matching contributions to the plan may be made at the discretion of the Company's Board. The Company made no contributions to the plan during the years ended December 31, 2016 and 2017.

17. Subsequent Events

The Company has completed an evaluation of all subsequent events after the audited balance sheet date of December 31, 2017 through the filing date of this Registration Statement on Form S-1 with the SEC, to ensure that these consolidated financial statements include appropriate disclosure of events both recognized in the

17. Subsequent Events (continued)

consolidated financial statements as of December 31, 2017, and events which occurred subsequently but were not recognized in the consolidated financial statements. The Company has concluded that no subsequent events have occurred that require disclosure, except as disclosed within these consolidated financial statements and except as described below.

(a) Subsequent Events Through April 6, 2018

On January 12, 2018, the Company entered into a lease agreement for office space located in Cambridge Massachusetts, United States, which expires in January 2023, with a landlord who is an affiliate of the landlord of the Company's current lease facility. In accordance with the lease agreement, the Company is required to maintain a security deposit of $209. In contemplation of this agreement, the Company terminated its existing lease agreement.

On January 19, 2018, the Company entered into a stock purchase agreement for the sale of 28,285,557 shares of Series B Preferred Stock for $2.1389 per share. The total gross proceeds received were $60,500. In addition, the Company issued 233,765 shares of Series B Preferred Stock to BioMarin as required by the Company's license agreement with BioMarin (Note 11). The rights and preferences of the Series B Preferred Stock are similar to the Company's Series A Preferred Stock, in that holders of outstanding Series B Preferred Stock have priority and preference to Series Seed Preferred Stock and common stock in the case of a liquidation or redemption event. The issue price of the Series B Preferred Stock is $2.1389 per share, and each share of Series B Preferred Stock is convertible into 0.242 shares of common stock.

In January 2018, the Company granted 753,789 stock options with an exercise price of $5.00 per share.

(b) Subsequent Events Through June 11, 2018

Reverse Stock Split

On June 1, 2018, the Board approved a 1-for-4.132 reverse stock split of the Company's common stock. The reverse stock split was approved by the stockholders on June 7, 2018 and became effective on June 8 2018. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. All share and per share data shown in the accompanying consolidated financial statements and related notes have been retroactively revised to reflect the reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of the Company's Preferred Stock were proportionately reduced and the respective conversion prices were proportionately increased.

2018 Stock Option and Incentive Plan

The AVROBIO, Inc. 2018 Stock Option and Incentive Plan (the 2018 Plan) was adopted by the Board June 1, 2018 and approved by stockholders on June 7, 2018 and will become effective upon the effectiveness of the Company's Registration Statement on Form S-1. The 2018 Plan will replace the 2015 Plan as the Board determined not to make additional awards under the 2015 Plan following the pricing of the Company's IPO. The 2018 Plan allows the compensation committee to make equity-based and cash-based incentive awards to our officers, employees, directors and other key persons (including consultants).

17. Subsequent Events (continued)

The Company initially reserved 616,300 shares of our common stock for the issuance of awards under the 2018 Plan. The 2018 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2019, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

2018 Employee Stock Purchase Plan

The AVROBIO, Inc. 2018 Employee Stock Purchase Plan (the ESPP) was adopted by the Board on June 1, 2018 and approved by stockholders on June 7, 2018 and will become effective upon the effectiveness of the Company's Registration Statement on Form S-1. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423(b) of the Code. The ESPP initially reserves and authorizes the issuance of up to a total of 223,200 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2019 and each January 1 thereafter through January 1, 2028, by the least of (i) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31; (ii) 1,115,700 shares or (iii) such number of shares as determined by the ESPP administrator. The number of shares reserved under the ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

Fourth Amended and Restated Certificate of Incorporation

On June 1, 2018, the Board also approved for filing immediately following the effectiveness of the Company's registration statement in connection with its IPO, the Fourth Amended and Restated Certificate of Incorporation, which shall, among other matters: (i) authorize 150,000,000 shares of common stock, $0.0001 par value and (ii) create 10,000,000 shares of undesignated preferred stock.

AVROBIO, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(amounts in thousands, except share and per share data)

	December 31, 2017	March 31, 2018	Pro Forma March 31, 2018
Assets			
Current assets:			
Cash and cash equivalents	$ 5,963	$ 57,928	$ 57,928
Prepaid expenses and other current assets	345	553	553
Total current assets	6,308	58,481	58,481
Property and equipment, net	349	604	604
Other assets	365	1,131	1,131
Total assets	$ 7,022	$ 60,216	$ 60,216
Liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity			
Current liabilities:			
Accounts payable	$ 527	$ 2,052	$ 2,052
Accrued expenses and other current liabilities	2,098	3,009	3,009
Total current liabilities	2,625	5,061	5,061
Warrant to purchase redeemable convertible preferred stock	35	47	—
Derivative liability	371	958	958
Deferred rent, net of current portion	126	161	161
Other long-term liability	500	—	—
Total liabilities	3,657	6,227	6,180
Commitments and contingencies (Note 13)			
Redeemable convertible preferred stock (Note 8)	26,500	87,500	—
Stockholders' (deficit) equity:			
Common stock, $0.0001 par value; 51,000,000 and 82,000,000 shares authorized as of December 31, 2017 and March 31, 2018, respectively; 2,581,474 shares issued as of December 31, 2017 and March 31, 2018; 2,305,173 and 2,335,926 shares outstanding as of December 31, 2017 and March 31, 2018, respectively; 17,901,687 shares issued and 17,656,139 shares outstanding as of March 31, 2018 (pro forma)	—	—	2
Additional paid-in capital	339	109	87,654
Accumulated deficit	(23,474)	(33,620)	(33,620)
Total stockholders' (deficit) equity	(23,135)	(33,511)	54,036
Total liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity	$ 7,022	$ 60,216	$ 60,216

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AVROBIO, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(amounts in thousands, except share and per share data)

	Three Months Ended March 31,	
	2017	2018
Operating expenses:		
Research and development	$ 1,434	$ 5,647
General and administrative	610	2,141
Total operating expenses	2,044	7,788
Loss from operations	(2,044)	(7,788)
Other income (expense):		
Interest income	4	158
Change in fair value of preferred stock warrant liability	—	(12)
Change in fair value of derivative liability	(32)	(587)
Other expense	(5)	(13)
Total other (expense) income, net	(33)	(454)
Net loss	$ (2,077)	$ (8,242)
Comprehensive loss	$ (2,077)	$ (8,242)
Reconciliation of net loss to net loss attributable to common stockholders:		
Net loss	$ (2,077)	$ (8,242)
Accretion of issuance costs on redeemable convertible preferred stock	(47)	(2,243)
Net loss attributable to common stockholders—basic and diluted	$ (2,124)	$ (10,485)
Net loss per share attributable to common stockholders—basic and diluted (Note 12)	$ (0.97)	$ (4.51)
Weighted-average number of common shares used in computing net loss per share attributable to common stockholders—basic and diluted	2,181,715	2,324,790
Pro forma net loss per share attributable to common stockholders—basic and diluted (Note 12)		$ (0.51)
Pro forma weighted-average number of common shares used in computing pro forma net income (loss) per share attributable to common stockholders—basic and diluted		16,187,901

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AVROBIO, INC.
CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' (DEFICIT) EQUITY
(unaudited)
(amounts in thousands, except share data)

	Series Seed Redeemable Convertible Preferred Stock		Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
Balance as of December 31, 2017	3,333,333	$ 1,500	31,450,499	$ 25,000	—	$ —	2,305,173	$ —	$ 339	$ (23,474)	$ (23,135)
Issuance of series B redeemable convertible preferred stock, net of issuance costs of $2,243	—	—	—	—	28,285,557	58,257	—	—	—	—	—
Issuance of series B redeemable convertible preferred stock to settled accrued liability of license cost	—	—	—	—	233,765	500	—	—	—	—	—
Vesting of restricted stock awards	—	—	—	—	—	—	30,753	—	—	—	—
Accretion of issuance costs related to redeemable convertible preferred stock	—	—	—	—	—	2,243	—	—	(339)	(1,904)	(2,243)
Stock-based compensation expense	—	—	—	—	—	—	—	—	109	—	109
Net loss	—	—	—	—	—	—	—	—	—	(8,242)	(8,242)
Balances as of March 31, 2018	3,333,333	1,500	31,450,499	25,000	28,519,322	61,000	2,335,926	—	109	(33,620)	(33,511)
Reclassification of warrants to purchase shares of redeemable convertible preferred stock into warrants to purchase common stock	—	—	—	—	—	—	—	—	47	—	47
Conversion of redeemable convertible preferred stock into common stock	(3,333,333)	(1,500)	(31,450,499)	(25,000)	(28,519,322)	(61,000)	15,320,213	2	87,498	—	87,500
Pro forma balance as of March 31, 2018	—	$ —	—	$ —	—	$ —	17,656,139	$ 2	$ 87,654	$ (33,620)	$ 54,036

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AVROBIO, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(amounts in thousands)

	Three Months Ended March 31,	
	2017	2018
Cash flows from operating activities:		
Net loss	$(2,077)	$ (8,242)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	31	109
Depreciation and amortization expense	2	21
Impairment loss of property and equipment	—	235
Amortization of deferred offering costs	—	11
Deferred rent expense	136	(8)
Change in fair value of preferred stock warrant liability	—	12
Change in fair value of derivative liability	32	587
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(105)	(189)
Other assets	(8)	(306)
Accounts payable	22	1,438
Accrued expenses and other current liabilities	(44)	352
Net cash used in operating activities	(2,011)	(5,980)
Cash flows from investing activities:		
Purchases of property and equipment	(55)	(263)
Net cash used in investing activities	(55)	(263)
Cash flows from financing activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	3,487	58,263
Payments of initial public offering costs	—	(55)
Net cash provided by financing activities	3,487	58,208
Net increase in cash and cash equivalents	1,421	51,965
Cash and cash equivalents at beginning of period	5,357	5,963
Cash and cash equivalents at end of period	$ 6,778	$57,928
Supplemental disclosure of non-cash investing and financing activities:		
Purchases of property and equipment included in accounts payable	$ 247	$ 88
Purchases of property and equipment paid for by landlord	$ —	$ 181
Property and equipment held for sale	$ —	$ 19
Deferred offering costs included in accrued expenses	$ —	$ 507
Accretion of issuance costs related to redeemable convertible preferred stock	$ 47	$ 2,240

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
Three Months Ended March 31, 2017 and 2018
(amounts in thousands, except share and per share data)

1. Nature of the Business

AVROBIO, Inc. (the "Company" or "AVROBIO") is a clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral gene therapies to treat rare diseases following a single dose.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Through March 31, 2018, the Company has funded its operations primarily with proceeds from the sale of series Seed redeemable convertible preferred stock (the "Series Seed Preferred Stock"), series A redeemable convertible preferred stock (the "Series A Preferred Stock") and series B redeemable convertible preferred stock (the "Series B Preferred Stock"), (the Series Seed Preferred Stock, the Series A Preferred Stock and the Series B Preferred Stock are collectively referred to as the "Preferred Stock"). The Company has incurred recurring losses since its inception, including net losses of $8,242 for the three months ended March 31, 2018. In addition, as of March 31, 2018, the Company had an accumulated deficit of $33,621. The Company expects to continue to generate operating losses for the near future. The future viability of the Company is dependent on its ability to raise additional capital to finance its operations. The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurance that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

The Company believes the cash and cash equivalents on hand as of March 31, 2018 of $57,928 will be sufficient to fund its operations and capital expenditure requirements through at least May 11, 2019.

2. Summary of Significant Accounting Policies

Basis of presentation

The accompanying condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

The unaudited condensed consolidated interim financial statements have been prepared on the same basis as the audited annual consolidated financial statements as of and for the year ended December 31, 2017, and, in the opinion of management, reflect all adjustments, consisting of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of March 31, 2018, and the results of its operations and its cash flows for the three months ended March 31, 2017 and 2018.

2. Summary of Significant Accounting Policies (continued)

The results for the three months ended March 31, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018, any other interim periods, or any future year or period. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2017, and the notes thereto, which are included elsewhere in the Company's confidentially submitted Registration Statement on Form S-1.

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the year ended December 31, 2017 included in the Company's Form S-1. Since the date of such consolidated financial statements, there have been no changes to the Company's significant accounting policies.

Unaudited Pro Forma Information

The accompanying unaudited pro forma condensed consolidated balance sheet and statement of redeemable convertible preferred stock and stockholders' (deficit) equity as of March 31, 2018 has been prepared to give effect, upon the closing of a qualified initial public offering ("IPO"), to the conversion of all outstanding shares of redeemable convertible preferred stock into 15,320,213 shares of common stock and the conversion of the outstanding warrant to purchase shares of redeemable convertible preferred stock as of March 31, 2018 into a warrant to purchase shares of common stock as if the Company's proposed IPO had occurred on March 31, 2018.

In the accompanying unaudited condensed consolidated statements of operations and comprehensive loss, the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2018 has been computed using the weighted-average number of common shares outstanding after giving pro forma effect to the conversion of all outstanding shares of redeemable convertible preferred stock into shares of common stock and the conversion of the outstanding warrant to purchase shares of redeemable convertible preferred stock as of March 31, 2018 into a warrant to purchase shares of common stock, as if such conversion had occurred at the beginning of the period presented or the date of original issuance, whichever is later. Accordingly, the effect of the accretion to redemption value of the redeemable convertible preferred stock has been excluded from the determination of pro forma basic and diluted loss per share attributable to common stockholders. Additionally, the changes in the fair value of the warrant to purchase redeemable convertible preferred stock has been excluded from the determination of pro forma basic and diluted net loss per share attributable to common stockholders as these instruments are not required be recorded at fair value once it becomes a warrant to purchase common stock.

Subsequent Event Considerations

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. Subsequent events have been evaluated as required. See Note 16.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

3. Fair Value of Financial Assets and Liabilities

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values as of December 31, 2017 and March 31, 2018, respectively:

	Fair Value Measurements as of December 31, 2017 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$ 5,684	$ —	$ —	$ 5,684
Restricted cash	24	—	—	24
	$ 5,708	$ —	$ —	$ 5,708
Liabilities:				
Derivative liability	$ —	$ —	$ 371	$ 371
Warrant to purchase redeemable convertible preferred stock	—	—	35	35
	$ —	$ —	$ 406	$ 406

	Fair Value Measurements as of March 31, 2018 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$57,459	$ —	$ —	$57,459
Restricted cash	24			24
	$57,483	$ —	$ —	$57,483
Liabilities:				
Derivative liability	$ —	$ —	$ 958	$ 958
Warrant to purchase redeemable convertible preferred stock	—	—	47	47
	$ —	$ —	$1,005	$ 1,005

During the three months ended March 31, 2017 and 2018, there were no transfers between Level 1, Level 2 and Level 3.

Valuation of the Warrant to Purchase Preferred Stock

The warrant to purchase preferred stock liability in the table above is composed of the fair value of a warrant to purchase shares of Series A Preferred Stock that was issued to a lender in connection with a loan and security agreement in 2017. The fair value of the warrant to purchase preferred stock was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

In order to determine the fair value of the warrant to purchase preferred stock, the Company utilizes available facts and circumstances to estimate the number of shares of Series A Preferred Stock for which the warrant will ultimately be exercisable. The Company then used the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value the warrant to purchase preferred stock. Estimates and

3. Fair Value of Financial Assets and Liabilities (continued)

assumptions impacting the fair value measurement include the fair value of the underlying shares of Series A Preferred Stock, the remaining contractual term of the warrant, risk-free interest rate, expected dividend yield and expected volatility of the price of the underlying preferred stock. The Company determined the fair value of the underlying preferred stock based on various valuation methodologies. The Company lacks company-specific historical and implied volatility information of its stock. Therefore, it estimates its expected stock volatility based on the historical volatility of publicly traded guideline companies for a term equal to the remaining contractual term of the warrant. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrant. The Company estimated no expected dividend yield based on the fact that the Company has never paid or declared dividends and does not intend to do so in the foreseeable future.

The assumptions that the Company used to determine the fair value of the warrant to purchase preferred stock are as follows:

	December 31, 2017	March 31, 2018
Remaining contractual term	9.48	9.23
Risk-free interest rate	2.40%	2.74%
Expected volatility	82.00%	82.00%
Expected dividend yield	0%	0%
Fair value of Series A Preferred Stock per share	$ 1.42	$ 1.88

The following table sets forth a summary of changes in the fair value of the Company's preferred stock warrant liability for which fair value is determined by Level 3 inputs:

	Warrant Liability
Balance as of December 31, 2017	$ 35
Change in fair value.	12
Balance as of March 31, 2018	$ 47

Valuation of Derivative

In January 2016, in connection with a license agreement entered into with University Health Network ("UHN"), and as part of the initial consideration for the license, the Company issued 1,161,665 shares of common stock to UHN pursuant to a stock purchase agreement (the "Stock Purchase Agreement"). The Stock Purchase Agreement contains a provision requiring the Company to make a cash payment to UHN of up to $2,000 if UHN's fully diluted ownership is reduced within specified percentages as part of an IPO by the Company. The Company concluded the anti-dilution feature represented a derivative instrument and should be measured at fair value, with changes in fair value recognized as a gain or loss to other income (expense), net in the consolidated statements of operations and comprehensive loss. The initial fair value of the derivative of $49 was recorded as research and development expense in January 2016.

On March 31, 2017 and 2018, the Company remeasured the fair value of the derivative, using current assumptions, resulting in an increase in fair value of $32 and $587, respectively, which was recorded in other expense in the accompanying condensed consolidated statements of operations and comprehensive loss for the three months ended March 31, 2017 and 2018. The Company will continue to re-measure the fair value of the liability at the end of each reporting period until the completion of an IPO.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

3. Fair Value of Financial Assets and Liabilities (continued)

The following table sets forth a summary of changes in the fair value of the Company's derivative liability for which fair value is determined by Level 3 inputs:

	Derivative Liability
Balance as of December 31, 2017	$ 371
Change in fair value	587
Balance as of March 31, 2018	$ 958

4. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31, 2017	March 31, 2018
Prepaid development costs	$ 163	$ 221
Prepaid rent	122	95
Other current assets	60	237
	$ 345	$ 553

5. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31, 2017	March 31, 2018
Laboratory and office equipment	$ 126	$ 395
Leasehold improvements	240	181
Computer equipment and software	28	53
	394	629
Less: Accumulated depreciation and amortization	(45)	(25)
	$ 349	$ 604

Depreciation and amortization expense for the three months ended March 31, 2017 and 2018 was $2 and $21, respectively.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

6. Accrued Expenses

Accrued expenses consisted of the following:

	December 31, 2017	March 31, 2018
Compensation and benefit costs	$ 794	$ 493
Short-term deferred rent	9	146
Research and development costs	831	1,363
Consulting and professional fees	267	532
Preferred stock issuance cost	85	6
Legal services fee	31	451
Other liabilities	81	18
	$ 2,098	$ 3,009

7. Loan Agreement and Warrant to Purchase Preferred Stock

On June 23, 2017, the Company entered into the Loan Agreement with a lender, which provided for the issuance of term loans of up to $10,000, subject to the achievement of various development and corporate milestones. Any outstanding principal amounts under the Loan Agreement will accrue interest at a floating per annum rate equal to the greater of 1% and the “prime rate,” as defined in the Loan Agreement, minus 3%.

Payments on the Loan Agreement are interest only, payable monthly in arrears, until November 1, 2018, which can be extended by six months if the third tranche is drawn. Thereafter, principal and interest amounts are repayable over a 30-month period, unless the third tranche is funded and the initial interest-only period is extended by six months, in which case principal and interest amounts are repayable over a 24-month period. As of March 31, 2018, the Company had not drawn down from the facility and $10,000 was available to the Company.

The Loan Agreement contains customary indemnification obligations and customary events of default. In the event of default by the Company under the Loan Agreement, the lender would be entitled to exercise their remedies thereunder, including the right to accelerate the debt, upon which the Company may be required to repay all amounts then outstanding under the Loan Agreement or the lender may take possession of the collateral securing the Loan Agreement. No events of default had occurred through March 31, 2018.

The Loan Agreement also includes certain restrictions on, among other things, the Company’s ability to incur additional indebtedness, change the name or location of its business, merge with or acquire other entities, pay dividends or make other distributions to holders of the Company’s capital stock, make certain investments, engage in transactions with affiliates, create liens, open new deposit accounts, sell assets or pay subordinated debt.

In connection with the Loan Agreement, the Company agreed to issue a warrant to the lender for the purchase of up to 188,702 shares of the Company’s Series A Preferred Stock with an exercise price of $0.7949 per share. The warrant expires on June 22, 2027. The warrant is initially exercisable for the purchase of up to 28,305 shares of Series A Preferred Stock and can become exercisable for up to an additional 160,397 shares of Series A Preferred Stock based on the amounts drawn under the Loan Agreement. On the issuance date of the warrant, the Company recorded a deferred financing cost and a liability for the warrant to purchase preferred stock in the Company’s consolidated balance sheet equal to the issuance-date fair value of the warrant. As of March 31, 2018, the warrant is exercisable for up to 28,305 shares of Series A Preferred Stock.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

7. Loan Agreement and Warrant to Purchase Preferred Stock (continued)

On the date of issuance, the fair value of the warrant was determined to be $18. The Company remeasured the liability associated with the warrant as of December 31, 2017 and March 31, 2018, and determined that the fair value of the preferred stock warrant liability was $35 and $47, respectively. The Company recognized a loss of $12 in the condensed consolidated statements of operations and comprehensive loss for the three months ended March 31, 2018, related to the change in fair value of the warrant.

8. Redeemable Convertible Preferred Stock

As of March 31, 2018, the authorized capital stock of the Company included 63,491,857 shares of $0.0001 par value preferred stock, of which 3,333,333 shares have been designated as Series Seed redeemable convertible preferred stock (the "Series Seed Preferred Stock), 31,639,202 shares have been designated as Series A redeemable convertible preferred stock (the "Series A Preferred Stock) and 28,519,322 shares have been designated as Series B redeemable convertible preferred stock (the "Series B Preferred Stock) (the Series Seed Preferred Stock, the Series A Preferred Stock and the Series B Preferred Stock are collectively referred to as the "Preferred Stock").

In January 2018, the Company issued and sold 28,519,322 shares of Series B Preferred Stock, at a price of $2.1389 per share, for total proceeds of $58,757, net of issuance costs of $2,243.

As of December 31, 2017 and March 31, 2018, the Preferred Stock consisted of the following:

	December 31, 2017				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series Seed preferred stock	3,333,333	3,333,333	$ 1,500,000	$ 1,500,000	806,711
Series A preferred stock	31,639,202	31,450,499	25,000,000	25,000,000	7,611,438
	34,972,535	34,783,832	$ 26,500,000	$ 26,500,000	8,418,149

	March 31, 2018				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series Seed preferred stock	3,333,333	3,333,333	$ 1,500,000	$ 1,500,000	806,711
Series A preferred stock	31,639,202	31,450,499	25,000,000	25,000,000	7,611,438
Series B preferred stock	28,519,322	28,519,322	61,000,000	61,000,000	6,902,064
	63,491,857	63,303,154	$ 87,500,000	$ 87,500,000	15,320,213

As of March 31, 2018, the rights and preferences of the holders of the Preferred Stock had not been changed since December 31, 2017, except for the following which were amended upon issuance of Series B Preferred Stock:

Liquidation Rights—

In the event of any voluntary or involuntary liquidation event, dissolution, winding up of the Company or upon the occurrence of certain events designated by a majority of the holders of the Preferred Stock to be a

8. Redeemable Convertible Preferred Stock (continued)

deemed liquidation event, each holder of the then outstanding Series A Preferred Stock and Series B Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of Series Seed Preferred Stock and common stock, an amount equal to the greater of (i) $0.7949 per share for Series A and $2.1389 per share for Series B Preferred Stock (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

After the payment of all preferential amounts to the holders of Series A Preferred Stock and Series B Preferred Stock, each holder of the then outstanding Series Seed Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of common stock, an amount equal to the greater of (i) $0.45 per share (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

After payments have been made in full to the holders of the Preferred Stock, then, to the extent available, the remaining amounts will be distributed among the holders of the shares of common stock, pro rata based on the number of shares held by each holder.

Conversion—

Each share of Preferred Stock is convertible into common stock, at any time, at the option of the holder, and without the payment of additional consideration, at the applicable conversion ratio then in effect for each series of Preferred Stock, initially set to be one-for-one, and subject to adjustment in accordance with anti-dilution provisions. In addition, each share of Preferred Stock will be automatically converted into common stock at the applicable conversion ratio then in effect for each series of Preferred Stock upon the earlier of a qualified IPO which results in net proceeds of at least $50,000 with the issuance price at least 1.5 times the original issue price of Series B Preferred Stock and the listing of the Company's common stock on the New York Stock Exchange or the Nasdaq Stock Market, or upon a vote of the holders of a majority of the outstanding Preferred Stock. As of March 31, 2018, each share of Preferred Stock was convertible into 0.242 shares of common stock and can be adjusted in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock.

The Company evaluated each series of its Preferred Stock and determined that each individual series is considered an equity host. In making this determination, the Company's analysis followed the whole instrument approach which compares an individual feature against the entire preferred stock instrument which includes that feature. The Company's analysis was based on a consideration of the economic characteristics and risks of each series of Preferred Stock. More specifically, the Company evaluated all of the stated and implied substantive terms and features, including: (1) whether the Preferred Stock included redemption features, (2) how and when any redemption features could be exercised, (3) whether the holders of Preferred Stock were entitled to dividends, (4) the voting rights of the Preferred Stock and (5) the existence and nature of any conversion rights.

As a result of the Company's conclusion that the Preferred Stock represents an equity host, the conversion feature of all series of Preferred Stock is considered to be clearly and closely related to the associated Preferred Stock host instrument. Accordingly, the conversion feature of all series of Preferred Stock is not considered an embedded derivative that requires bifurcation.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

8. Redeemable Convertible Preferred Stock (continued)

The Company accounts for potential beneficial conversion features at the time of issuance. The Company's common stock into which each series of the Company's Preferred Stock is convertible had an estimated fair value less than the effective conversion prices of the Preferred Stock at the time of each of the issuances of Preferred Stock. Therefore, there was no intrinsic value on the respective commitment dates. In addition, the Company considered the other features included within the Preferred Stock and determined that none of the other features required bifurcation and separate accounting.

Redemption—

The Series Seed Preferred Stock, Series A Preferred Stock and Series B Preferred Stock are redeemable at $0.45 per share, $0.7949 per share and $2.1389 per share, respectively, (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any declared but unpaid dividends, on or after January 19, 2023 at the written election of at least a majority of the holders of Preferred Stock voting together as a single class. The redemption is paid in three annual installments. No bifurcation of the redemption feature was required as the feature does not contain the characteristics of a derivative instrument.

As the Preferred Stock is redeemable upon the passage of time, the Preferred Stock has been classified outside of permanent equity. The Company has elected to record the changes in the redemption value immediately as they occur.

9. Common Stock

As of March 31, 2018, the authorized capital stock of the Company included 82,000,000 shares of common stock, $0.0001 par value, respectively. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth above.

Each share of common stock entitles the holder to one vote, together with the holders of the Preferred Stock, on all matters submitted to the stockholders for a vote. The holders of the Preferred Stock, voting as a single class, are entitled to elect three directors of the Company. The holders of common stock, together with the holders of the Preferred Stock and voting as a single class, are entitled to elect two directors of the Company. The holders of common stock and of any other class or series of voting stock (including the Preferred Stock), voting together as a single class, are entitled to elect the remaining directors of the Company. Common stockholders are entitled to receive dividends, as may be declared by the Board, if any, subject to the preferential dividend rights of the Preferred Stock. Through March 31 2018, no cash dividends have been declared or paid.

9. Common Stock (continued)

At December 31, 2017 and March 31, 2018, the Company has reserved the following shares of common stock for future issuance:

	December 31, 2017	March 31, 2018
Shares reserved for Series Seed Preferred Stock outstanding	806,711	806,711
Shares reserved for Series A Preferred Stock outstanding	7,611,438	7,611,438
Shares reserved for Series B Preferred Stock outstanding	—	6,902,064
Shares reserved for vesting of restricted stock awards	276,301	245,548
Shares reserved for exercise of outstanding stock options	1,034,961	1,788,750
Shares reserved for issuance under the 2015 Stock Option and Grant Plan	143,717	58,472
Total shares of authorized common stock reserved for future issuance	9,873,128	17,412,983

10. Stock-Based Compensation

Amended and Restated 2015 Stock Option and Grant Plan

The Company's Amended and Restated 2015 Stock Option and Grant Plan, (the "2015 Plan") provides for the Company to issue restricted stock awards and restricted stock units, or to grant incentive stock options or non-statutory stock options. Incentive stock options may be granted only to the Company's employees including officers and members of the Board who are also employees. Restricted stock awards and restricted stock units and non-statutory stock options may be granted to employees, members of the Board, outside advisors, and consultants of the Company.

The total number of common shares that may be issued under the 2015 Plan was 2,008,564 shares as of March 31, 2018, of which 58,472 shares remained available for future grant.

Shares that expire, are terminated, surrendered or canceled under the 2015 Plan without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future awards.

The 2015 Plan is administered by the Board. Equity awards granted to employees and members of the Board typically vest over four years.

During the three months ended March 31, 2018, the Company granted options to purchase 753,789 shares of common stock to employees and members of the Board. No options were granted by the Company during the three months ended March 31, 2017.

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

10. Stock-Based Compensation (continued)

Stock Option Valuation

The assumptions that the Company used to determine the grant-date fair value of stock options granted to employees and members of the Board were as follows, presented on a weighted-average basis:

	Three Months Ended March 31, 2018
Expected option life (years)	6.07
Risk-free interest rate	2.72%
Expected volatility	84.00%
Expected dividend yield	—%

The following table summarizes the Company's stock option activity for the three months ended March 31, 2018:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2017	1,034,961	$ 0.78	8.94	$ 3,427
Granted	753,789	$ 5.00	9.96	
Exercised	—			
Cancelled or forfeited	—			
Outstanding as of March 31, 2018	1,788,750	$ 2.56	9.22	$ 11,075
Exercisable as of March 31, 2018	222,905	$ 0.68	8.22	$ 1,802
Unvested as of March 31, 2018	1,565,845	$ 2.83	9.37	$ 9,273

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the underlying stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the estimated fair value of the Company's common stock at March 31, 2018.

The weighted-average grant-date fair value of the Company's stock options granted during the three months ended March 31, 2018 was $6.99.

Restricted Common Stock

The following table summarizes the Company's restricted common stock activity for the three months ended March 31, 2018:

	Number of Shares	Weighted-Average Grant Date Fair Value
Issued and unvested as of December 31, 2017	276,301	$ 0.42
Vested	(30,753)	0.42
Forfeited, canceled or expired	—	—
Issued an unvested as of March 31, 2018	245,548	0.42

AVROBIO, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (CONTINUED)
Three Months Ended March 31, 2017 and 2018

10. Stock-Based Compensation (continued)

The total fair value of restricted common stock vested during the three months ended March 31, 2017 and 2018 was $9 and $13, respectively.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows:

	Three Months Ended March 31,	
	2017	2018
Research and development	$ 17	$ 36
General and administrative	14	73
Total stock based compensation expense	$ 31	$ 109

As of March 31, 2018, total unrecognized compensation cost related to the unvested stock-based awards was $6,043, which is expected to be recognized over a weighted-average period of 3.81 years.

11. License Agreements

Agreements with UHN

Fabry License Agreement—

On January 27, 2016, the Company entered into an agreement with UHN, pursuant to which UHN granted the Company an option to enter into an exclusive license under the UHN intellectual property related to Fabry disease in accordance with the pre-negotiated licensing terms. On November 4, 2016, the Company exercised its option and entered into a license agreement with UHN, pursuant to which UHN granted the Company an exclusive worldwide license under certain intellectual property rights and a non-exclusive worldwide license under certain know-how, in each case subject to certain retained rights, to develop, commercialize and sell products for use in the treatment of Fabry disease. In addition, for three years following the execution of the agreement, UHN granted the Company an exclusive option to obtain a license under certain improvements to the licensed intellectual property rights as well as an option to negotiate a license under certain other improvements.

Under this agreement, the Company paid an option fee of CAD $20, an upfront license fee of CAD $75, plus the annual license maintenance fee for the first year. Thereafter, the Company is also required to pay UHN future annual license maintenance fees until the first sale of a licensed product in certain markets. The Company is also obligated to make future milestone payments in an aggregate amount of up to CAD $2,450 upon the achievement of specified milestones as well as royalties on a country-by-country basis of a low to mid-single digit percentage of annual net sales of licensed products and a lower single-digit royalty percentage in certain circumstances. Additionally, the Company has agreed to pay a low double-digit royalty percentage of all sublicensing revenue. The agreement requires the Company to meet certain performance milestones within specified timeframes.

UHN may terminate the agreement if the Company fails to meet these performance milestones despite using commercially reasonable efforts and the Company is unable to reach agreement with UHN on revised timeframes. The Company’s royalty obligation expires on a licensed product-by-licensed product and

11. License Agreements (continued)

country-by-country basis upon the latest to occur of the expiration or termination of the last valid claim under the licensed intellectual property rights in such country, the tenth anniversary of the first commercial sale of such licensed product in such country and the expiration of any applicable regulatory exclusivity in such country.

Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products. UHN can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event that the Company fails to obtain or maintain insurance. Either the Company or UHN may terminate the license agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company can voluntarily terminate the agreement with prior notice to UHN.

For the three months ended March 31, 2017 and 2018, no upfront fee and maintenance fees were incurred related to Fabry license agreement.

Interleukin 12 License Agreement—

On January 27, 2016, the Company entered into an exclusive license agreement with UHN, pursuant to which UHN granted the Company a license to certain patent rights for the commercial development, manufacture, distribution and use of any products or processes resulting from development of those patent rights related to Interleukin 12. Upon execution of this agreement, the Company paid an upfront license fee of CAD $264. In addition, as part of the initial consideration for the license, the Company issued to UHN 1,161,665 shares of the Company's common stock. The fair value of the shares issued to UHN of $480 and the upfront fee was expensed upon the execution of the agreement. In addition, the Company agreed to pay UHN up to $2,000 upon the closing of an IPO if certain criteria are met. This obligation is considered a derivative instrument and was initially recorded at fair value of $49. The Company is also required to pay UHN future annual license maintenance fees of CAD $50 on each anniversary of the effective date of the license agreement prior to expiration or termination and potential future milestone payments of up to CAD $19,275 upon the achievement of specified clinical and regulatory milestones. The Company also agreed to pay UHN royalties of a low single digit percentage of net sales of licensed products sold by the Company. If the Company grants any sublicense rights under the license agreement, the Company has agreed to pay UHN a low double-digit royalty percentage of any sublicense income received by the Company.

The agreement requires the Company to meet certain due diligence requirements based upon specified milestones. The agreement expires on the later of the date the last patent rights expire in the last country or ten years from the date of first sale. UHN can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event that the Company fails to obtain or maintain insurance. The Company can voluntarily terminate the agreement with prior notice to UHN. Either the Company or UHN may terminate the license agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time.

For the three months ended March 31, 2017 and 2018, the Company recorded research and development expense related to this agreement with UHN of $151 and $41, respectively, which consists of upfront fees, the fair value of the shares and derivative instrument issued to UHN, and license maintenance fees and development milestone payments.

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Three Months Ended March 31, 2017 and 2018

11. License Agreements (continued)

Agreement with BioMarin Pharmaceutical Inc. ("BioMarin")

On August 31, 2017, the Company entered into a license agreement with BioMarin, pursuant to which BioMarin granted the Company an exclusive worldwide license under certain intellectual property rights owned or controlled by BioMarin to develop, commercialize and sell products for use in the treatment of Pompe disease. As consideration for this agreement, the Company paid an upfront license fee of $500 in cash and issued 233,765 shares of Series B Preferred Stock to BioMarin at the time of our Series B Preferred Stock financing in January 2018. Both the upfront cash payment of $500 and the value of the shares Series B Preferred Stock issued of $500 were recorded as research and development expense during the year ended December 31, 2017. The Company is also obligated to make future milestone payments of up to $13,000 upon the achievement of certain specified milestones and agreed to pay BioMarin royalties of a low single-digit percentage of net sales of licensed products sold by the Company or its affiliates covered by patent rights in a relevant country. No upfront fees related to the license were recorded for the three months ended March 31, 2018.

Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products throughout the world. BioMarin and the Company can terminate the agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company may terminate the agreement at will upon written notice to BioMarin. BioMarin has the right to terminate the agreement upon the Company's bankruptcy or insolvency, or in the event of any challenge or opposition to the licensed patent rights or related actions brought by the Company or its affiliates or sublicensees, or if the Company, its affiliates or sublicensees knowingly assist a third-party in challenging or otherwise opposing the licensed patent rights, except as required under a court order or subpoena.

Agreement with GenStem Therapeutics, Inc. ("GenStem")

On October 2, 2017, the Company entered into a license agreement with GenStem, pursuant to which GenStem granted the Company an exclusive worldwide license, subject to certain retained rights, under certain intellectual property rights owned or controlled by GenStem to develop, commercialize and sell products for use in the treatment of cystinosis. Under this agreement, the Company paid an upfront license fee of $1,000 and is required to make payments upon completion of certain milestones up to an aggregate of $16,000. The Company also agreed to pay GenStem a tiered mid to high single-digit royalty percentage on annual net sales of licensed products as well as a low double-digit percentage of sublicense income received from certain third party licensees. The Company's royalty obligation expires on a licensed product-by-licensed product and country-by-country basis on the eleventh anniversary of the first commercial sale of such licensed product in such country or the expiration of the last valid claim under the licensed patent rights covering such licensed product in such country, whichever is later. Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products throughout the world. GenStem and the Company can terminate the agreement in the event of a material breach by the other party and failure to cure such breach within a certain period of time. The Company may terminate the agreement at will upon the specified prior written notice to GenStem. No upfront fees related to the license were recorded for the three months ended March 31, 2018.

Agreement with Lund University Rights Holders

On November 17, 2016, the Company entered into a license agreement with affiliates of Lund University, along with certain other relevant rights holders that may be added from time to time, pursuant to which such

11. License Agreements (continued)

rights holders granted to the Company an exclusive worldwide license, subject to certain retained rights, under certain intellectual property rights to develop, commercialize and sell products in any and all uses relevant to Gaucher disease. As consideration for the license, the Company is required to make payments in connection with the achievement of certain milestones up to an aggregate of $550. The agreement expires on the latest of (i) the twentieth anniversary of the end of a certain research project the Company is funding pursuant to an agreement with Lund University, (ii) the expiration of the term of any patent filed on the licensed rights that covers a licensed product, (iii) the expiration of any applicable marketing exclusivity right and (iv) such time that neither the Company nor any sublicensees, partners or contractors are commercializing a licensed product. Either the Company or the rights holders acting together may terminate the license agreement if the other such party commits a material breach and fails to cure such breach within a certain period of time, or if the other party enters into liquidation, becomes insolvent, or enters into composition or statutory reorganization proceedings. No upfront fees related to the license were recorded for the three months ended March 31, 2017 and 2018.

12. Net Loss per Share and Unaudited Pro Forma Net Loss per Share

For purposes of the diluted net loss per share calculation, stock options, unvested restricted stock, the warrant to purchase shares of Series A Preferred Stock and Preferred Stock are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, the weighted-average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same.

The following potentially dilutive common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended March 31,	
	2017	2018
Options to purchase common stock	599,439	1,788,750
Restricted common stock	388,735	245,548
Redeemable convertible preferred stock (as converted to common stock)	4,155,742	15,320,213
Warrants to purchase redeemable convertible preferred stock (as converted to common stock)	—	6,850

Unaudited Pro Forma Net Loss per Share Attributable to Common Stockholders

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2018 has been prepared to give effect to adjustments arising upon the closing of a qualified IPO. The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders does not include the effects of the accretion of Preferred Stock to redemption value or the change in fair value of the warrant to purchase shares of Series A Preferred Stock because the calculation gives effect to the conversion of shares of Preferred Stock outstanding as of March 31, 2018 into common stock and the conversion of the warrant to purchase shares of Series A Preferred Stock outstanding as of March 31, 2018 into a warrant to purchase shares of common stock, as if such conversion had occurred at the beginning of the period presented or the date of original issuance, whichever is later.

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12. Net Loss per Share and Unaudited Pro Forma Net Loss per Share (continued)

A reconciliation of pro forma net loss and the pro forma weighted-average number of common shares used in computing pro forma basic and diluted net loss per share applicable to common stockholders is as follows:

	Three Months Ended March 31, 2018
Numerator:	
Net loss attributable to common stockholders	$ (10,485)
Accretion of issuance cost on redeemable convertible preferred	2,243
Change in fair value of preferred stock warrant liability	12
Pro forma net loss attributable to common stockholders	$ (8,230)
Denominator:	
Weighted-average number of common shares used in computing net loss per share attributable to common stockholders—basic and diluted	2,324,790
Pro forma adjustment to reflect assumed conversion of redeemable convertible preferred stock into common stock	13,863,111
Pro forma weighted-average number of common shares used in computing pro forma net loss per share attributable to common stockholders—basic and diluted	16,187,901
Pro forma net loss per share attributable to common stockholders—basic and diluted	$ (0.51)

13. Commitments and Contingencies

Lease Agreements

On January 12, 2018, the Company entered into a lease agreement for office space located in Cambridge, Massachusetts. The lease agreement expires in January 2023, with a landlord who is an affiliate of the landlord of the Company's current lease facility. The annual lease payments are subject to a 3% increase each year. The Company recognizes rent expense on a straight-line basis over the lease period and has recorded deferred rent for rent expense incurred but not yet paid. The Company received a tenant incentive allowance of $181 in 2018. Such incentive allowance is being amortized as a reduction of rent expense on a straight-line basis over the lease period. In accordance with the lease agreement, the Company is required to maintain a security deposit of $209, which was recorded in other assets. In contemplation of this agreement, the Company terminated its existing lease agreement.

The Company recorded rent expense of $57 and $171 during the three months ended March 31, 2017 and 2018, respectively.

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the three months ended March 31, 2017 and 2018, and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

Other

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at December 31, 2017 and March 31, 2018, or royalties on future

13. Commitments and Contingencies (continued)

sales of specified products. No milestone or royalty payments under these agreements are expected to be payable in the immediate future.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company agrees to indemnify, hold harmless, and to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third-party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

14. Related Party Transactions

UHN

In connection with the Company's entry into a license agreement with UHN on January 27, 2016, the Company issued UHN 1,161,665 shares of its common stock. As a result of the issuance of common stock, UHN owned 9.65% and 5.90% of the Company's fully diluted equity as of December 31, 2017 and March 31, 2018, respectively. Upon the closing of the sale of shares of common stock in an IPO, if UHN's fully-diluted percentage ownership of the Company is reduced within a range of specified percentages, then the Company is obligated to pay UHN an amount up to $2,000. See Note 3 for further discussion on the accounting treatment for this provision.

During the three months ended March 31, 2017 and 2018, the Company recognized $151 and $41 respectively, of research and development expense related to the license agreements with UHN.

The Company recorded research and development expenses of $7 and $3 related to participation on the scientific advisory board and consulting services performed by a member of the Board who is affiliated with UHN during the years ended December 31, 2016 and 2017, respectively. As of December 31, 2016 and 2017, there was $7 and $10, respectively, included in accrued expenses on the Company's consolidated balance sheets related to these services.

For the years ended December 31, 2016 and 2017 the Company recorded expenses of $13 and $86, respectively, related to consulting services provided by an entity affiliated with an officer of the Company and a member of the Board. The entity is also a shareholder of the Company and owned 2.25% and 1.00% of the Company's fully diluted equity as of December 31, 2016 and 2017, respectively.

Others

For the three months ended March 31, 2017, the Company recorded expenses of $15 related to services provided by an entity affiliated with a member of the Board and the use of office space. The lease was terminated in February 2017.

15. Recently Issued Accounting Pronouncements

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) I. Accounting for Certain Financial Instruments*

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15. Recently Issued Accounting Pronouncements (continued)

with Down Round Features II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception. Part I applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. For public entities, the amendments in Part I of this update are effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. For all other entities, the amendments in Part I of this update are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted for all entities, including adoption in an interim period. The Company is currently evaluating the impact that the adoption of ASU 2017-11 will have on its consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. The standard is effective for annual periods beginning after December 15, 2017. Early adoption is permitted, including adoption in any interim period, for (1) public business entities for reporting periods for which financial statements have not yet been issued and (2) all other entities for reporting periods for which financial statements have not yet been made available for issuance. The Company is currently evaluating the impact that the adoption of ASU 2017-09 will have on its consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash* ("ASU 2016-18"), which requires restricted cash to be presented with cash and cash equivalents on the statement of cash flows and disclosure of how the statement of cash flows reconciles to the balance sheet if restricted cash is shown separately from cash and cash equivalents on the balance sheet. For public entities, the standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. For all other entities, the standard is effective for annual periods beginning after December 15, 2018, including interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2016-18 will have on its consolidated financial statements.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments* ("ASU 2016-15"), to address diversity in practice in how certain cash receipts and cash payments are presented and classified in the statement of cash flows. For public entities, the standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. For all other entities, the standard is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. The Company is currently evaluating the impact that the adoption of ASU 2016-15 will have on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed

15. Recently Issued Accounting Pronouncements (continued)

purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less may be accounted for similar to existing guidance for operating leases today. ASU 2016-02 supersedes the previous leases standard, ASC 840, Leases. For public entities, not-for-profit entities and an employee benefit plan that files financial statements with the U.S. Securities and Exchange Commission (SEC), the standard is effective for public entities for annual periods beginning after December 15, 2018 including interim periods within those fiscal years. For all other entities, the standard is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted.

In September 2017, the FASB issued ASU No. 2017-13, *Revenue Recognition (Topic 605), Revenue from Contracts with Customers (Topic 606), Leases (Topic 840), and Leases (Topic 842)*, which provides additional clarification and implementation guidance related to ASU 2016-02 and has the same effective date and transition requirements as ASU 2016-02. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its consolidated financial statements.

16. Subsequent Events

The Company has completed an evaluation of all subsequent events after the unaudited balance sheet date of March 31, 2018 through the filing date of this Registration Statement on Form S-1 with the SEC, to ensure that these consolidated financial statements include appropriate disclosure of events both recognized in the consolidated financial statements as of March 31, 2018, and events which occurred subsequently but were not recognized in the consolidated financial statements. The Company has concluded that no subsequent events have occurred that require disclosure, except as disclosed within these unaudited condensed consolidated financial statements.

Reverse Stock Split

On June 1, 2018, the Board approved a 1-for-4.132 reverse stock split of the Company's common stock. The reverse stock split was approved by the stockholders on June 7, 2018 and became effective on June 7, 2018. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. All share and per share data shown in the accompanying unaudited condensed consolidated financial statements and related notes have been retroactively revised to reflect the reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of the Company's Preferred Stock were proportionately reduced and the respective conversion prices were proportionately increased.

2018 Stock Option and Incentive Plan

The AVROBIO, Inc. 2018 Stock Option and Incentive Plan (the 2018 Plan) was adopted by the Board June 1, 2018 and approved by stockholders on June 7, 2018 and will become effective upon the effectiveness of the Company's Registration Statement on Form S-1. The 2018 Plan will replace the 2015 Plan as the Board determined not to make additional awards under the 2015 Plan following the pricing of the Company's IPO. The

16. Subsequent Events (continued)

2018 Plan allows the compensation committee to make equity-based and cash-based incentive awards to our officers, employees, directors and other key persons (including consultants).

The Company initially reserved 616,300 shares of our common stock for the issuance of awards under the 2018 Plan. The 2018 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2019, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

2018 Employee Stock Purchase Plan

The AVROBIO, Inc. 2018 Employee Stock Purchase Plan (the ESPP) was adopted by the Board on June 1, 2018 and approved by stockholders on June 7, 2018 and will become effective upon the effectiveness of the Company's Registration Statement on Form S-1. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423(b) of the Code. The ESPP initially reserves and authorizes the issuance of up to a total of 223,200 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2019 and each January 1 thereafter through January 1, 2028, by the least of (i) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31; (ii) 1,115,700 shares or (iii) such number of shares as determined by the ESPP administrator. The number of shares reserved under the ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

Fourth Amended and Restated Certificate of Incorporation

On June 1, 2018, the Board also approved for filing immediately following the effectiveness of the Company's registration statement in connection with its IPO, the Fourth Amended and Restated Certificate of Incorporation, which shall, among other matters: (i) authorize 150,000,000 shares of common stock, $0.0001 par value and (ii) create 10,000,000 shares of undesignated preferred stock.

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5,247,958 Shares

Common Stock

PROSPECTUS

MORGAN STANLEY
COWEN
WELLS FARGO SECURITIES
WEDBUSH PACGROW

June 20, 2018