

PROSPECTUS

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**Up to \$50,000,000**

**Common Stock**

We have entered into a Sales Agreement with Cowen and Company, LLC, or Cowen, relating to shares of our common stock, par value \$0.0001 per share, offered by this prospectus. In accordance with the terms of the Sales Agreement, we may offer and sell shares of our common stock having an aggregate offering price of up to \$50,000,000 from time to time through Cowen, acting as sales agent. Sales of the shares of common stock, if any, may be made on The Nasdaq Global Select Market at market prices and such other sales as agreed upon by us and Cowen, as the case may be.

Our common stock is listed on The Nasdaq Global Select Market under the symbol "AVRO." On June 27, 2019, the closing price of our common stock, as reported on The Nasdaq Global Select Market, was \$15.36 per share.

Sales of our common stock, if any, under this prospectus will be made in sales deemed to be "at the market offerings" as defined in Rule 415 promulgated under the Securities Act of 1933, as amended, or the Securities Act. Cowen is not required to sell any specific number or dollar amount of securities, but will act as our sales agent using commercially reasonable efforts to sell on our behalf all of the shares of common stock requested to be sold by us, consistent with their normal trading and sales practices, on mutually agreed terms between Cowen and us. There is no arrangement for funds to be received in any escrow, trust or similar arrangement.

Cowen will be entitled to compensation at a fixed commission rate of 3% of the gross sales price per common stock sold under the Sales Agreement. See "Plan of Distribution" beginning on page SA-27 for additional information regarding the compensation to be paid to Cowen. In connection with the sale of the common stock on our behalf, Cowen will be deemed to be an "underwriter" within the meaning of the Securities Act and the compensation of Cowen will be deemed to be underwriting commissions or discounts. We have also agreed to provide indemnification and contribution to Cowen with respect to certain liabilities, including liabilities under the Securities Act or the Securities Exchange Act of 1934, as amended, or the Exchange Act.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

**Investing in our common stock involves a high degree of risk. You should review carefully the risks and uncertainties referenced under the heading "[Risk Factors](#)" on page SA-8 of this prospectus and in the documents that are incorporated by reference into 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.

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**Cowen**

The date of this Prospectus is July 10, 2019

**TABLE OF CONTENTS**

	<b>Page</b>
<a href="#">About this Prospectus</a>	SA-1
<a href="#">Prospectus Summary</a>	SA-3
<a href="#">The Offering</a>	SA-6
<a href="#">Risk Factors</a>	SA-8
<a href="#">Cautionary Statement Regarding Forward-Looking Statements</a>	SA-12
<a href="#">Use of Proceeds</a>	SA-14
<a href="#">Dividend Policy</a>	SA-15
<a href="#">Dilution</a>	SA-16
<a href="#">Material U.S. Federal Income Tax Considerations for Non-U.S. Holders</a>	SA-18
<a href="#">Description of Capital Stock</a>	SA-22
<a href="#">Plan of Distribution</a>	SA-27
<a href="#">Legal Matters</a>	SA-29
<a href="#">Experts</a>	SA-29
<a href="#">Where You Can Find More Information</a>	SA-29
<a href="#">Incorporation by Reference</a>	SA-29

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We are responsible for the information contained and incorporated by reference in this prospectus and in any related free writing prospectus we prepare or authorize. We have not authorized anyone to give you any other information, and we take no responsibility for any other information that others may give you. If you are in a jurisdiction where offers to sell, or solicitations of offers to purchase, the securities offered by this documentation are unlawful, or if you are a person to whom it is unlawful to direct these types of activities, then the offer presented in this document does not extend to you. The information contained in this document speaks only as of the date of this document, unless the information specifically indicates that another date applies. Our business, financial condition, results of operations and prospects may have changed since those dates.

## ABOUT THIS PROSPECTUS

This prospectus is part of a shelf registration statement on Form S-3 that we filed with the Securities and Exchange Commission ("SEC"). Under the shelf registration process, we may offer shares of our common stock having an aggregate offering price of up to \$50,000,000 from time to time under this prospectus at prices and on terms to be determined by market conditions at the time of the offering.

If the information contained in this prospectus differs or varies from the information contained in any document incorporated by reference herein that was filed with the SEC before the date of this prospectus, you should rely on the information set forth in this prospectus. If any statement in one of these documents is inconsistent with a statement in another document having a later date (for example, a subsequently filed document deemed incorporated by reference in this prospectus), the statement in the document having the later date modifies or supersedes the earlier statement. Any statement so modified or superseded will not be deemed, except as so modified or superseded, to constitute a part of this prospectus.

You should rely only on the information contained or incorporated by reference in this prospectus. We have not, and the sales agent has not, authorized anyone to provide you with information that is in addition to or different from that contained or incorporated by reference in this prospectus or contained in any permitted free writing prospectuses we have authorized for use in connection with this offering. We and the sales agent take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide.

The information contained in this prospectus and the documents incorporated by reference herein is accurate only as of their respective dates, regardless of the time of delivery of any such document or the time of any sale of our common stock. Our business, financial condition, results of operations and prospects may have changed since those dates. It is important for you to read and consider all information contained or incorporated by reference in this prospectus in making your investment decision. You should read this prospectus, as well as the documents incorporated by reference herein, the additional information described under the section titled "Where You Can Find More Information" and "Incorporation of Certain Documents by Reference" in this prospectus and any free writing prospectus that we have authorized for use in connection with this offering, before investing in our common stock.

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to any document that is incorporated by reference in this prospectus were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreements, and should not be deemed to be a representation, warranty or covenant to you. Moreover, such representations, warranties or covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

We use various trademarks and trade names in our business, including without limitation our corporate name and logo. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, 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. This prospectus and the documents incorporated by reference herein also contain estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and

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[Table of Contents](#)

the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where such offers and sales are permitted. The distribution of this prospectus and the offering of our common stock in certain jurisdictions may be restricted by law. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of our common stock and the distribution of this prospectus outside the United States. This prospectus does not constitute, and may not be used in connection with, an offer to sell, or a solicitation of an offer to buy, any securities offered by this prospectus by any person in any jurisdiction in which it is unlawful for such person to make such an offer or solicitation.

As used in this prospectus, unless the context otherwise requires, references to the “company,” “we,” “us” and “our” refer to AVROBIO, Inc. together with its consolidated subsidiaries.

## PROSPECTUS SUMMARY

*This summary highlights selected information about us and this offering and does not contain all of the information that you should consider before investing in our securities. Before investing in our common stock, you should carefully read the information contained and incorporated by reference in this prospectus, including the sections titled "Risk Factors" and the financial statements and accompanying notes.*

### Company Overview

We are a Phase 2 clinical stage gene therapy company focused on developing potentially curative *ex vivo* lentiviral-based gene therapies to treat patients with rare diseases following a single dose treatment regimen. Our gene therapies employ hematopoietic stem cells that are harvested 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 is designed to transform stem cells from patients into therapeutic products, has the potential to provide curative benefit for a range of diseases. Our initial focus is on a group of rare genetic diseases referred to as lysosomal storage diseases, some of which today are primarily managed with enzyme replacement therapies, or ERTs. These lysosomal storage diseases have well-understood biologies, identified patient populations, established standards of care yet with significant unmet needs, and represent large market opportunities with approximately \$4.0 billion in worldwide net sales in 2018.

Since its first clinical use in 2003, lentiviral-based gene therapy has been observed to be well-tolerated in third parties' clinical trials for rare diseases such as beta thalassemia, adrenoleukodystrophy, or ALD, metachromatic leukodystrophy, or MLD, and ADA-SCID. The use of *ex vivo* lentiviral-based gene therapies was initially restricted primarily to the most acutely severe diseases where the risks of the typical requirement for ablating the patients' bone marrow, which significantly impairs those patients' immune systems, had a clinically justifiable risk/benefit profile due to the novelty of the approach and the severity of the diseases. To date, over 200 patients have been treated with lentiviral-based gene therapies in third parties' and our rare disease clinical trials, and we believe the technology can be developed for other serious conditions based on a rigorous risk/benefit assessment. The ablation procedure, also known as the conditioning regimen, is an inherent part of the *ex vivo* gene therapy treatment procedure and is administered prior to the gene therapy. The more intensive the conditioning regimen, the greater the risk of toxicity. However, we believe the risk of toxicity can be managed through utilization of therapeutic drug monitoring, or TDM. We are refining our approach to conditioning to use a single-agent, myeloablative conditioning regimen administered in conjunction with TDM, which is designed to permit patients to receive appropriate levels of conditioning to promote long-term engraftment while seeking to lessen toxicity. Such an approach could potentially allow the conditioning regimen to be performed during a limited hospital stay or potentially through an outpatient procedure, on a case-by-case basis as may be directed by the patient's physician. We believe our approach, utilizing a single myeloablative conditioning agent, coupled with TDM, has the potential to extend the reach of our gene therapies to a broad range of diseases as first-line therapies. Our goal is to broaden the applicability of lentiviral-based gene therapy by initially targeting diseases with tractable gene mutations, well-understood biology and that benefit from the sustained systemic delivery of an active protein.

We are initially targeting rare lysosomal storage 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 currently primarily managed by bi-weekly, multi-hour infusions with ERTs that seek to exogenously replace the missing enzyme. However, most ERTs 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 halt the progression of these diseases while also providing significant health economic advantages.

#### **plato: Our Commercial-Scale Platform**

Since forming our company, we have centered our efforts on developing first-line gene therapies, based on preceding academic approaches. While we believe these approaches have been adequate for our development programs to date, key to our strategy is to continuously improve our technology and production processes and to leverage these improvements across our gene therapies. We have designed plato to serve as our commercial-scale platform for our anticipated future worldwide commercialization activities, and our platform improvements already include differentiated features such as cryopreserved end-product and streamlined three-day manufacturing. We are continuing our transition towards implementing our plato platform, including moving our cell processing to an automated, closed system, transitioning to utilizing our proprietary four-plasmid-produced lentiviral vector, which we refer to as LV2, and a refined approach to conditioning utilizing busulfan that is administered with TDM. If successfully implemented, we believe our plato platform will represent a significant advance in our industry towards achieving the quality and scale required for global commercialization of gene therapies.

#### **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. We completed our initial public offering in June 2018. Our executive offices are located at One Kendall Square, Building 300, Suite 201, Cambridge, Massachusetts 02139 and our telephone number is (617) 914-8420. Our website address is [www.avrobio.com](http://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. Our common stock trades on the Nasdaq Global Select Market under the symbol "AVRO".

#### **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, or the JOBS Act. 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;

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[Table of Contents](#)

- 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 our initial public offering in June 2018; (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, are not subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” if the market value of our common stock held by non-affiliates is below \$250 million (or \$700 million if our annual revenue is less than \$100 million) as of June 30 in any given year, which would allow us to take advantage of many of the same exemptions from disclosure requirements.

## THE OFFERING

Common stock offered by us	Shares of our common stock having an aggregate offering price of up to \$50,000,000.
Common stock to be outstanding immediately after this offering	Up to 27,331,970 shares (as more fully described in the notes following this table), assuming sales of 3,255,208 shares of our common stock in this offering at an offering price of \$15.36 per share, which was the last reported sale price of our common stock on The Nasdaq Global Select Market on June 27, 2019. The actual number of shares issued will vary depending on how many shares of our common stock we choose to sell and the prices at which such sales occur.
Manner of offering	“At the market offering” that may be made from time to time on The Nasdaq Global Select Market or other existing trading market for our common stock through our sales agent, Cowen. See “ <a href="#">Plan of Distribution</a> ” on page SA-27 of this prospectus.
Use of proceeds	Our management will retain broad discretion regarding the allocation and use of the net proceeds. We currently intend to use the net proceeds from this offering primarily to fund activities relating to the advancement of our product candidates, and for other general corporate purposes, including, but not limited to, research and development costs, potential strategic acquisitions of complementary businesses, services or technologies, expansion of our technology infrastructure and capabilities, working capital and capital expenditures. See “ <a href="#">Use of Proceeds</a> ” on page SA-14 of this prospectus.
Risk factors	Investing in our common stock involves significant risks. See “ <a href="#">Risk Factors</a> ” on page SA-8 of this prospectus and under similar headings in the documents incorporated by reference into this prospectus for a discussion of the factors you should carefully consider before deciding to invest in our common stock.
The Nasdaq Global Select Market symbol	“AVRO”

All information in this prospectus related to the number of shares of our common stock to be outstanding immediately after this offering is based on 24,076,762 shares of our common stock



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[Table of Contents](#)

outstanding as of March 31, 2019. The number of shares outstanding as of March 31, 2019 as used throughout this prospectus, unless otherwise indicated, excludes:

- 2,986,048 shares of common stock issuable upon the exercise of stock options outstanding as of March 31, 2019 at a weighted-average exercise price of \$10.94 per share;
- 2,300 shares of common stock issuable upon the vesting of restricted stock units outstanding as of March 31, 2019;
- 528,134 shares of common stock reserved for future issuance under our 2018 Stock Option and Incentive Plan; and
- 461,266 shares of common stock reserved for the future issuance under our 2018 Employee Stock Purchase Plan.

Unless otherwise stated, all information contained in this prospectus assumes no exercise of stock options after March 31, 2019 and reflects an assumed public offering price of \$15.36, which was the last reported sale price of our common stock on The Nasdaq Global Select Market on June 27, 2019.

## RISK FACTORS

*Investing in our common stock involves a high degree of risk. Before making an investment decision, you should carefully consider the risks described below and in our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q or Current Reports on Form 8-K, as well as any amendments thereto reflected in subsequent filings with the SEC, each of which are incorporated by reference in this prospectus, and all of the other information in this prospectus, including our financial statements and related notes incorporated by reference herein. If any of these risks are realized, or if other risks not currently known to us materialize, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the trading price of our common stock could decline and you could lose part or all of your investment. Additional risks and uncertainties that are not yet identified or that we currently believe to be immaterial may also materially harm our business, financial condition, results of operations and prospects and could result in a complete loss of your investment.*

### Risks Related To This Offering and 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 offering price.***

Our stock price is likely to be volatile. Since our IPO in June 2018, the trading price of our common stock has ranged from \$11.85 to \$53.70. 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 price at which you purchase shares. 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;

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## [Table of Contents](#)

- 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 be sustained.***

Prior to our initial public offering in June 2018, or our IPO, there had been no public market for our common stock. Although our common stock is listed on The Nasdaq Global Select Market, an active trading market for our shares may never be sustained. If an active market for our common stock 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. Although we have obtained research coverage from certain analysts, there can be no assurance that analysts will continue to cover us or provide favorable 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.***

Based on shares outstanding as of May 31, 2019, our executive officers, directors, five percent stockholders and their affiliates beneficially owned approximately 49% of our outstanding voting stock. 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 affairs. 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

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[Table of Contents](#)

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 you may purchase our shares 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 our IPO; (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 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 reduced or 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.

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” if the market value of our common stock held by non-affiliates is below \$250 million (or \$700 million if our annual revenue is less than \$100 million) as of June 30 in any given year, which would allow us to take advantage of many of the same exemptions from disclosure requirements.

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[Table of Contents](#)

***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 as adjusted 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 \$9.69 per share, based on the assumed offering price of \$15.36 per share, which is the closing price of our common stock on June 27, 2019, and our as adjusted net tangible book value as of March 31, 2019. Further, based on these assumptions, investors purchasing shares of common stock in this offering will contribute approximately 19.7% of the total amount invested by stockholders since our inception, but will own only approximately 11.9% of the shares of common stock outstanding. For information on how the foregoing amounts were calculated, see "Dilution."

***Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.***

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that such sales may have on the prevailing market price of our common stock. In addition, the sale of substantial amounts of our common stock could adversely impact its price. As of March 31, 2019, we had outstanding 24,076,762 shares of our common stock and options to purchase 2,986,048 shares of our common stock (of which 673,946 were exercisable as of that date). The sale or the availability for sale of a large number of shares of our common stock in the public market could cause the price of our common stock to decline.

***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 prior loan facility with Silicon Valley Bank restricted our ability to pay any dividends or making any distributions on account of our capital stock, and we may enter into agreements in the future with similar restrictions. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

## CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This prospectus and the information and documents incorporated by reference herein, contain forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act. Any statements about our expectations, beliefs, plans, objectives, assumptions or future events or performance are not historical facts and may be forward-looking. These statements are often, but are not always, made through the use of words or phrases such as “may,” “will,” “could,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “projects,” “potential,” “continue,” and similar expressions, or the negative of these terms, or similar expressions. Accordingly, these statements involve estimates, assumptions, risks and uncertainties which could cause actual results to differ materially from those expressed in them. Any forward-looking statements are qualified in their entirety by reference to the factors discussed throughout this prospectus and the documents incorporated by reference herein, and in particular those factors referenced in the section “Risk Factors.”

This prospectus, any related free writing prospectus and the information and documents incorporated by reference herein contain forward-looking statements that are based on our management’s belief and assumptions and on information currently available to our management. These statements relate to future events or our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements 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 existence or absence of side effects or other properties relating to our product candidates which could delay or prevent their regulatory approval, limit their commercial potential, or result in significant negative consequences following any potential marketing approval;
- 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, technology and plato platform, including our transition to a proprietary four-plasmid-produced lentiviral vector, or LV2, and our use of busulfan as a conditioning regimen administered through therapeutic drug monitoring, or TDM;
- 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 use of cryopreservation and implementation of a closed, automated manufacturing 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;

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## [Table of Contents](#)

- the scope of protection we and/or our licensors are able to establish and maintain for intellectual property rights covering our product candidates;
- our financial performance;
- our ability to retain the continued service of our key professionals and to identify, hire and retain additional qualified professionals;
- 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, including without limitation recently enacted tax reform legislation;
- our expectations regarding the time during which we are an emerging growth company under the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act; and
- other risks and uncertainties, including those listed under the caption “Risk Factors”

These forward-looking statements are neither promises nor guarantees of future performance due to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those indicated by these forward-looking statements, including, without limitation the risk factors and cautionary statements described in other documents that we file from time to time with the SEC, specifically under “Item 1A: Risk Factors” and elsewhere in our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q or Current Reports on Form 8-K, and the section of the prospectus titled “Risk Factors.”

The forward-looking statements in this prospectus, any related free writing prospectus and the documents incorporated by reference represent our views as of their respective dates. 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 assume no obligation to update or revise any forward-looking statements 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 dates on which they were made.

This prospectus, any related free writing prospectus and the documents incorporated by reference also contain estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

## USE OF PROCEEDS

We may issue and sell shares of our common stock having aggregate sales proceeds of up to \$50,000,000 from time to time. The amount of proceeds from this offering will depend upon the number of shares of our common stock sold and the market price at which they are sold. There can be no assurance that we will be able to sell any shares under or fully utilize the sales agreement with Cowen as a source of financing. Therefore, the actual total public offering amount, commissions and proceeds to us, if any, are not determinable at this time.

We intend to use any net proceeds from this offering primarily to fund activities relating to the advancement of our product candidates, and for other general corporate purposes. General corporate purposes may include research and development costs, including the conduct of one or more clinical trials and process development and manufacturing of our product candidates, potential strategic acquisitions of complementary businesses, services or technologies, expansion of our technology infrastructure and capabilities, working capital and capital expenditures and other general corporate purposes. We may temporarily invest the net proceeds in a variety of capital preservation instruments, including investment grade, interest bearing instruments and U.S. government securities, until they are used for their stated purpose. We have not determined the amount of net proceeds to be used specifically for such purposes. As a result, management will retain broad discretion over the allocation of net proceeds.



## **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.

## DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted to the extent of the difference between the public offering price per share of our common stock in this offering and the as adjusted net tangible book value per share of our common stock immediately after this offering. Our net tangible book value as of March 31, 2019 was approximately \$106.8 million, or approximately \$4.44 per share of common stock. Net tangible book value per share represents the amount of total tangible assets (total assets less intangible assets) less total liabilities, divided by the number of shares of our common stock outstanding as of March 31, 2019.

Dilution in net tangible book value per share to new investors participating in this offering represents the difference between the amount per share paid by purchasers in this offering and the as adjusted net tangible book value per share of our common stock immediately after giving effect to this offering. After giving effect to the assumed sale of 3,255,208 shares of our common stock in the aggregate amount of approximately \$50,000,000 at an assumed offering price of \$15.36 per share, the last reported sale price of our common stock on June 27, 2019, and after deduction of commissions and estimated aggregate offering expenses payable by us, our as adjusted net tangible book value as of March 31, 2019 would have been approximately \$155 million, or approximately \$5.67 per share of common stock. This represents an immediate increase in net tangible book value of \$1.23 per share of common stock to our existing stockholders and an immediate dilution in net tangible book value of \$9.69 per share of common stock to investors participating in this offering at the assumed offering price.

Dilution per share to new investors is determined by subtracting as adjusted net tangible book value per share after this offering from the assumed public offering price per share paid by new investors. The following table illustrates this per share dilution:

Assumed offering price per share		\$15.36
Net tangible book value per share as of March 31, 2019	\$4.44	
Increase in net tangible book value per share attributable to new investors attributable to this offering	<u>1.23</u>	
As adjusted net tangible book value per share as of March 31, 2019, after giving effect to this offering		<u>5.67</u>
Dilution in net tangible book value per share to new investors participating in this offering		<u>\$ 9.69</u>

The table above assumes for illustrative purposes that an aggregate of 3,255,208 shares of our common stock are sold at a price of \$15.36 per share, the last reported sale price of our common stock on The Nasdaq Global Select Market on June 27, 2019, for aggregate gross proceeds of approximately \$50,000,000. The shares sold in this offering, if any, will be sold from time to time at various prices. An increase of \$1.00 per share in the price at which the shares are sold from the assumed offering price of \$15.36 per share shown in the table above, assuming all of our common stock in the aggregate amount of approximately \$50,000,000 is sold at that price, would result in an increase to our adjusted net tangible book value per share after the offering to \$5.79 and an increase in the dilution in net tangible book value per share to new investors in this offering to \$10.57, after deducting commissions and estimated offering expenses payable by us. A decrease of \$1.00 per share in the price at which the shares are sold from the assumed offering price of \$15.36 per share shown in the table above, assuming all of our common stock in the aggregate amount of approximately \$50,000,000 is sold at that price, would result in a decrease to our adjusted net tangible book value per share after the offering to \$5.56 and a decrease in the dilution in net tangible book value per share to

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[Table of Contents](#)

new investors in this offering to \$8.80, after deducting commissions and estimated offering expenses payable by us. This information is supplied for illustrative purposes only, and will adjust based on the actual offering prices, the actual number of shares that we offer and sell in this offering and other terms of each sale of shares in this offering.

The information above and in the foregoing table is based upon 24,076,762 shares of our common stock outstanding as of March 31, 2019. The information above and in the foregoing table excludes as of March 31, 2019:

- 2,986,048 shares of common stock issuable upon the exercise of stock options outstanding as of March 31, 2019 at a weighted-average exercise price of \$10.94 per share;
- 2,300 shares of common stock issuable upon the vesting of restricted stock units outstanding as of March 31, 2019;
- 528,134 shares of common stock reserved for future issuance under our 2018 Stock Option and Incentive Plan; and
- 461,266 shares of common stock reserved for the future issuance under our 2018 Employee Stock Purchase Plan.

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

## MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS

The following discussion is a summary of certain material U.S. federal income tax considerations for 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 U.S. state, local or non-U.S. taxes, the alternative minimum tax, the rules regarding qualified small business stock within the meaning of Section 1202 of the Code, the Medicare tax on net investment income or any other aspect of any U.S. federal tax other than the income tax. 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" as defined in Section 897(I)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds;
- persons that have a functional currency other than the U.S. dollar;

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## [Table of Contents](#)

- 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 ownership and disposition of our common stock.

### **Distributions on Our Common Stock**

As described in the “[Risk Factors](#)” section above, we do not anticipate paying any cash dividends on our capital stock in the foreseeable future. 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 “[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 “[FATCA](#),” a non-U.S. holder generally will not be subject to any U.S. federal income or withholding tax

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[Table of Contents](#)

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 or 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.

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[Table of Contents](#)

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.

**FATCA**

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally impose a U.S. federal withholding tax at a rate of 30% on payments of dividends on 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. Such withholding may also apply to gross proceeds from the sale or other disposition of our common stock, although under recently proposed U.S. Treasury Regulations, no withholding would apply to such gross proceeds. The preamble to the proposed regulations specifies that taxpayers (including withholding agents) are permitted to rely on the proposed regulations pending finalization. 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.

## DESCRIPTION OF CAPITAL STOCK

*The following description of our common stock and preferred stock summarizes the material terms and provisions of the common stock that we may offer under this prospectus. The following description of our capital stock does not purport to be complete and is subject to, and qualified in its entirety by, our certificate of incorporation and bylaws, which are exhibits to the registration statement of which this prospectus forms a part, and by applicable law. The terms of our common stock and preferred stock may also be affected by Delaware law.*

### General

Our authorized capital stock consists 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 are undesignated.

As of March 31, 2019, 24,076,762 shares of our common stock were outstanding and held by 15 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

Our board of directors has 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. As of June 30, 2019, no shares of preferred stock were outstanding.

### Stock Options

As of March 31, 2019, there were outstanding options to purchase an aggregate of 2,986,048 shares of our common stock.

### Registration Rights

As of June 30, 2019, the holders of 4,756,478 shares of our common stock are 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, which



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[Table of Contents](#)

was subsequently converted into common stock in connection with our initial public offering in June 2018, or our IPO. 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***

The holders of 4,756,478 shares of our common stock 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 our IPO and (iii) 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 includes 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.

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[Table of Contents](#)

***Board Composition and Filling Vacancies***

Our amended and restated certificate of incorporation provides 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 also provides 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 provides 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 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 limits 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 establishes 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 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.

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[Table of Contents](#)

***Undesignated Preferred Stock***

Our amended and restated certificate of incorporation provides 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 provides 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 contains 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. On December 19, 2018, the Court of Chancery of the State of Delaware issued a decision declaring that such federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court are ineffective and invalid under Delaware law. On January 17, 2019, the decision was appealed to the Delaware Supreme Court. While the Delaware Supreme Court recently dismissed the appeal on jurisdictional grounds, we expect that the appeal will be re-filed after the Court of Chancery issues a final judgment. Unless and until the Court of Chancery's decision is reversed by the Delaware Supreme Court or otherwise abrogated, we do not intend to enforce our federal forum selection provision designating the District of Massachusetts as the exclusive forum for Securities Act claims. In the event that the Delaware Supreme Court affirms the Court of Chancery's decision or otherwise determines that federal forum selection provisions are invalid, the Company's Board of Directors intends to amend promptly our amended and restated bylaws to remove our federal forum selection bylaw provision. As a result of the Court of Chancery's decision or a decision by the Supreme Court of Delaware affirming the Court of Chancery's decision, we may incur

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[Table of Contents](#)

additional costs associated with our federal forum selection bylaw provision, which could have an adverse effect on our business, financial condition and results of operations.

**Section 203 of the Delaware General Corporation Law**

We are 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**

Our common stock is listed on The Nasdaq Global Select Market under the trading symbol "AVRO."

**Transfer Agent and Registrar**

The transfer agent and registrar for our common stock is 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.

## PLAN OF DISTRIBUTION

We have entered into a sales agreement with Cowen, under which we may issue and sell from time to time up to \$50,000,000 of our common stock through Cowen as our sales agent. Sales of our common stock, if any, will be made at market prices by any method that is deemed to be an "at the market" offering as defined in Rule 415 under the Securities Act, including sales made directly on The Nasdaq Global Select Market or any other trading market for our common stock. If authorized by us in writing, Cowen may purchase shares of our common stock as principal.

Cowen will offer our common stock subject to the terms and conditions of the sales agreement on a daily basis or as otherwise agreed upon by us and Cowen. We will designate the maximum amount of common stock to be sold through Cowen on a daily basis or otherwise determine such maximum amount together with Cowen. Subject to the terms and conditions of the sales agreement, Cowen will use its commercially reasonable efforts to sell on our behalf all of the shares of common stock requested to be sold by us. We may instruct Cowen not to sell common stock if the sales cannot be effected at or above the price designated by us in any such instruction. Cowen or we may suspend the offering of our common stock being made through Cowen under the sales agreement upon proper notice to the other party. We and Cowen each have the right, by giving written notice as specified in the sales agreement, to terminate the sales agreement in either party's sole discretion at any time.

The aggregate compensation payable to Cowen as sales agent equals 3.0% of the gross sales price of the shares sold through Cowen pursuant to the sales agreement. We have also agreed to reimburse Cowen up to \$50,000 of Cowen's actual outside legal expenses incurred by Cowen in connection with this offering, and for certain other expenses, including Cowen's FINRA counsel fees in an amount up to \$10,000. We estimate that the total expenses of the offering payable by us, excluding commissions payable to Cowen under the sales agreement, will be approximately \$250,000.

The remaining sales proceeds, after deducting any expenses payable by us and any transaction fees imposed by any governmental, regulatory, or self-regulatory organization in connection with the sales, will equal our net proceeds for the sale of such common stock.

Cowen will provide written confirmation to us following the close of trading on The Nasdaq Global Select Market on each day in which common stock is sold through it as sales agent under the sales agreement. Each confirmation will include the number of shares of common stock sold through it as sales agent on that day, the volume weighted average price of the shares sold, the percentage of the daily trading volume and the net proceeds to us.

We will report at least quarterly the number of shares of common stock sold through Cowen under the sales agreement, the net proceeds to us and the compensation paid by us to Cowen in connection with the sales of common stock.

Settlement for sales of common stock will occur, unless the parties agree otherwise, on the second trading day following the date on which any sales were made in return for payment of the net proceeds to us. There is no arrangement for funds to be received in an escrow, trust or similar arrangement.

In connection with the sales of our common stock on our behalf, Cowen will be deemed to be an "underwriter" within the meaning of the Securities Act, and the compensation paid to Cowen will be deemed to be underwriting commissions or discounts. We have agreed in the sales agreement to provide indemnification and contribution to Cowen against certain liabilities, including liabilities under the Securities Act. As sales agent, Cowen will not engage in any transaction that stabilizes our common stock.

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[Table of Contents](#)

Our common stock is listed on The Nasdaq Global Select Market and trades under the symbol "AVRO." The transfer agent of our common stock is Computershare Trust Company, N.A.

Cowen and/or its affiliates have provided, and may in the future provide, various investment banking and other financial services for us for which services they have received and, may in the future receive, customary fees.

## LEGAL MATTERS

Certain legal matters in connection with this offering and the validity of the securities offered by this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, MA. Cowen and Company, LLC is being represented in connection with this offering by Ropes & Gray LLP, Boston, Massachusetts.

## EXPERTS

The consolidated financial statements of AVROBIO, Inc. appearing in AVROBIO, Inc.'s Annual Report (Form 10-K) for the year ended December 31, 2018, have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon included therein, and incorporated herein by reference. Such financial statements are, and audited financial statements to be included in subsequently filed documents will be, incorporated herein in reliance upon the reports of Ernst & Young LLP pertaining to such financial statements (to the extent covered by consents filed with the Securities and Exchange Commission) given on the authority of such firm as experts in accounting and auditing.

## WHERE YOU CAN FIND MORE INFORMATION

This prospectus is part of a registration statement on Form S-3 that we have filed with the SEC. This prospectus, filed as part of the registration statement, does not contain all the information set forth in the registration statement and its exhibits and schedules, portions of which have been omitted as permitted by the rules and regulations of the SEC. For further information about us, we refer you to the registration statement and to its exhibits and schedules. Certain information in the registration statement has been omitted from this prospectus in accordance with the rules of the SEC.

We are subject to the reporting and information requirements of the Exchange Act and, in accordance therewith, file annual, quarterly and special reports, proxy statements and other information with the SEC. These documents also may be accessed through the SEC's electronic data gathering, analysis and retrieval system, or EDGAR, via electronic means, including the SEC's home page on the Internet ([www.sec.gov](http://www.sec.gov)). Written requests for such copies should be directed to Investor Relations, AVROBIO, Inc., One Kendall Square, Building 300, Suite 201, Cambridge, Massachusetts 02139, and our website is located at [www.avrobio.com](http://www.avrobio.com).

We have the authority to designate and issue more than one class or series of stock having various preferences, conversion and other rights, voting powers, restrictions, limitations as to dividends, qualifications, and terms and conditions of redemption. See "Description of Capital Stock." We will furnish a full statement of the relative rights and preferences of each class or series of our stock which has been so designated and any restrictions on the ownership or transfer of our stock to any shareholder upon request and without charge. Information contained on our website is not incorporated by reference into this prospectus and, therefore, is not part of this prospectus or any accompanying prospectus supplement.

## INCORPORATION BY REFERENCE

The SEC allows us to incorporate by reference the information and reports we file with it, which means that we can disclose important information to you by referring you to these documents. The

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## [Table of Contents](#)

information incorporated by reference is an important part of this prospectus, and information that we file later with the SEC will automatically update and supersede the information already incorporated by reference. We are incorporating by reference the documents listed below, which we have already filed with the SEC, and any future filings we make with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act, including all filings made after the date of this prospectus, except as to any portion of any future report or document that is not deemed filed under such provisions, until we sell all of the securities:

- our Annual Report on [Form 10-K](#) for the year ended December 31, 2018, filed with the SEC on March 25, 2019;
- the information specifically incorporated by reference into our Annual Report on Form 10-K for the year ended December 31, 2018 from our definitive proxy statement on [Schedule 14A](#) (other than information furnished rather than filed), which was filed with the SEC on April 26, 2019;
- our Quarterly Report on [Form 10-Q](#) for the quarter ended March 31, 2019, filed with the SEC on May 13, 2019;
- our Current Report on Form 8-K filed with the SEC on [January 7, 2019](#), our Current Report on Form 8-K/A filed with the SEC on [January 11, 2019](#) and our Current Reports on Form 8-K filed with the SEC on [January 22, 2019](#), [February 6, 2019](#), [April 29, 2019](#) and [June 12, 2019](#) (in each case, except for information contained therein which is furnished rather than filed); and
- the description of our common stock contained in our registration statement on [Form 8-A](#) filed with the SEC on June 18, 2018, including any amendments or reports filed for the purposes of updating this description.

We incorporate by reference any future filings made with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act between the date of this prospectus and the termination of the offering.

Notwithstanding the foregoing, unless specifically stated to the contrary, information that we furnish (and that is not deemed “filed” with the SEC) under Items 2.02 and 7.01 of any Current Report on Form 8-K, including the related exhibits under Item 9.01, is not incorporated by reference into this prospectus or the registration statement of which this prospectus is a part.

Any statement contained in a document that is incorporated by reference will be modified or superseded for all purposes to the extent that a statement contained in this prospectus, or in any other document that is subsequently filed with the SEC and incorporated by reference into this prospectus, modifies or is contrary to that previous statement. Any statement so modified or superseded will not be deemed a part of this prospectus, except as so modified or superseded. Since information that we later file with the SEC will update and supersede previously incorporated information, you should look at all of the SEC filings that we incorporate by reference to determine if any of the statements in this prospectus or in any documents previously incorporated by reference have been modified or superseded.

Upon request, either orally or in writing, we will provide, without charge, to each person, including any beneficial owner, to whom a copy of this prospectus is delivered, a copy of the documents incorporated by reference into this prospectus but not delivered with the prospectus. You may request a copy of these filings, and any exhibits we have specifically incorporated by reference as an exhibit in this prospectus, at no cost by writing us at the following address: Investor Relations, AVROBIO, Inc., One Kendall Square, Building 300, Suite 201, Cambridge, Massachusetts 02139, or via telephone at (617) 914-8420.

This prospectus is part of a registration statement we filed with the SEC. We have incorporated exhibits into this registration statement. You should read the exhibits carefully for provisions that may be important to you.



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[Table of Contents](#)

You should rely only on the information incorporated by reference or provided in this prospectus or any prospectus supplement. We have not authorized anyone to provide you with different information. We are not making an offer of these securities in any state where the offer is not permitted. You should not assume that the information in this prospectus or in the documents incorporated by reference is accurate as of any date other than the date on the front of this prospectus or those documents.

**Up to \$50,000,000**



**Common Stock**

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**PROSPECTUS**

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**Cowen**

July 10, 2019

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