

**Prospectus Supplement  
(to Prospectus dated June 7, 2018)****\$150,000,000**

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We have entered into an Open Market Sale Agreement<sup>SM</sup> (the “sales agreement”) with Jefferies LLC (the “Agent”), relating to the sale of shares of our common stock offered by this prospectus supplement and the accompanying 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 \$150 million from time to time through the Agent acting as sales agent, at our discretion.

Our common stock is listed on the Nasdaq Capital Market under the symbol “ABEO.” On August 16, 2018, the last reported sale price of our common stock on the Nasdaq Capital Market was \$13.70 per share.

Sales of our common stock, if any, under this prospectus supplement and the accompanying prospectus may 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 (“the Securities Act”) including sales made directly on or through the Nasdaq Capital Market, the existing trading market for our common stock, sales made to or through a market maker other than on an exchange or otherwise, in negotiated transactions at market prices prevailing at the time of sale or at prices related to such prevailing market prices, and/or any other method permitted by law. The Agent is not required to sell any specific number or dollar amount of securities but will act as our sales agent using commercially reasonable efforts consistent with its normal trading and sales practices, on mutually agreed terms between the Agent and us. Our common stock to which this prospectus supplement relates will be sold through the Agent on any given day. There is no arrangement for funds to be received in any escrow, trust or similar arrangement.

The compensation to the Agent for sales of common stock sold pursuant to the sales agreement will be 3.0% of the gross proceeds from each such sale. In connection with the sale of the common stock on our behalf, the Agent may be deemed to be an “underwriter” within the meaning of the Securities Act, and the compensation of the Agent may be deemed to be underwriting commissions or discounts. We have also agreed to provide indemnification and contribution to the Agent with respect to certain liabilities, including liabilities under the Securities Act.

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**Investing in our securities involves significant risks. Please read the information contained in or incorporated by reference under the heading “Risk Factors” beginning on page [S-4](#) of this prospectus supplement, and under similar headings in other documents filed after the date hereof and incorporated by reference into this prospectus supplement and the accompanying prospectus.**

**Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus supplement or the accompanying prospectus. Any representation to the contrary is a criminal offense.**

Jefferies LLC

The date of this prospectus supplement is August 17, 2018

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## ABOUT THIS PROSPECTUS SUPPLEMENT

This prospectus supplement and the accompanying prospectus relate to the offering of our common stock. You should read this prospectus supplement, the accompanying prospectus, the documents incorporated by reference into this prospectus supplement and the accompanying prospectus, and any free writing prospectus that we may authorize for use in connection with this offering, in their entirety before making an investment decision. You should also read and consider the information in the documents to which we have referred you in the section of this prospectus supplement entitled "Where You Can Find More Information; Incorporation by Reference." These documents contain important information that you should consider when making your investment decision.

This document is in two parts. The first part is this prospectus supplement, which describes the specific terms of the offering of the common stock and also adds to and updates information contained in the accompanying prospectus and the documents incorporated by reference into this prospectus supplement and the accompanying prospectus. The second part, the accompanying prospectus, including the documents incorporated by reference into the accompanying prospectus, provides more general information, some of which may not apply to this offering. Generally, when we refer to this prospectus, we are referring to the combined document consisting of this prospectus supplement and the accompanying prospectus. To the extent there is a conflict between the information contained in this prospectus supplement, on the one hand, and the information contained in the accompanying prospectus or in any document incorporated by reference into the accompanying prospectus that was filed with the Securities and Exchange Commission ("SEC") before the date of this prospectus supplement, on the other hand, you should rely on the information in this prospectus supplement. If any statement in one of these documents is inconsistent with a statement in another document having a later date, the statement in the document having the later date modifies or supersedes the earlier statement.

We are responsible for the information contained in, or incorporated by reference into, this prospectus supplement, the accompanying prospectus and in any free writing prospectus that we may authorize for use in connection with this offering. We have not, and the Agent has not, authorized any other person to provide you with different information, and neither we nor the Agent takes any responsibility for any other information that others may give you.

We are not, and the Agent is not, making an offer to sell or soliciting an offer to buy our common stock in any jurisdiction in which an offer or solicitation is not authorized or in which the person making that offer or solicitation is not qualified to do so or to anyone to whom it is unlawful to make an offer or solicitation.

You should assume that the information appearing in this prospectus supplement, the accompanying prospectus, the documents incorporated by reference into this prospectus supplement and the accompanying prospectus, and in any free writing prospectus that we may authorize for use in connection with this offering, is accurate only as of the date of those respective documents. Our business, financial condition, results of operations and prospects may have changed since those dates.

Unless the context otherwise requires or as otherwise expressly stated, references in this prospectus to the terms "the Company," "Abeona," "we," "our" and "us" or other similar terms mean Abeona Therapeutics Inc. and its subsidiaries, unless we state otherwise or the context indicates otherwise.

## SUMMARY

*This summary highlights information contained elsewhere or incorporated by reference in this prospectus supplement and the accompanying prospectus. This summary does not contain all of the information that you should consider before deciding to invest in our common stock. You should read this entire prospectus supplement and the accompanying prospectus carefully, including the "Risk Factors" section contained in this prospectus supplement, our financial statements and the related notes thereto and the other documents incorporated by reference in this prospectus supplement and the accompanying prospectus.*

### **Overview**

We are a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Our lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV NAGLU), an AAV based gene therapy for Sanfilippo syndrome type B (MPS IIIB). We are also developing ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, we are developing a proprietary vector platform, AIM™, for next generation product candidates.

### **Company Information**

We were incorporated in 1974. On October 24, 2014, we changed our name to PlasmaTech Biopharmaceuticals, Inc., and on June 19, 2015, we changed our name to Abeona Therapeutics Inc. to reflect our broader rare disease commitment. Our principal executive office is located at 1330 Avenue of the Americas, 33<sup>rd</sup> Floor, New York, NY 10019. Our website address is [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com). We do not incorporate by reference into this prospectus supplement the information on our website, and you should not consider it as part of this prospectus supplement.

**THE OFFERING**

Common Stock Offered	Shares of our common stock having an aggregate offering price of up to \$150 million.
Manner of Offering	“At the market offering” that may be made from time to time through the Agent, as sales agent. See “Plan of Distribution” on page <a href="#">S-7</a> of this prospectus supplement for a more complete description of the manner of offering.
Sales Agent	Jefferies LLC.
Use of Proceeds	We expect to use the net proceeds of this offering, if any, for working capital and general corporate purposes, including research and development expenses and general and administrative expenses. See “Use of Proceeds” on page <a href="#">S-5</a> of this prospectus supplement for a more complete description of the intended use of proceeds from this offering.
Risk Factors	Investing in our securities involves significant risks. Please read the information contained in or incorporated by reference under the heading “Risk Factors” beginning on page <a href="#">S-4</a> of this prospectus supplement, and under similar headings in other documents filed after the date hereof and incorporated by reference into this prospectus supplement and the accompanying prospectus.
Nasdaq Capital Market symbol	ABEO.

## RISK FACTORS

*An investment in our securities involves risks. We urge you to consider carefully the risks described below, and in the documents incorporated by reference in this prospectus supplement and the accompanying prospectus, before making an investment decision, including those risks identified under "Item IA. Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2017, which is incorporated by reference in this prospectus supplement and which may be amended, supplemented or superseded from time to time by other reports that we subsequently file with the SEC. Additional risks, including those that relate to any particular securities we offer, may be included in a future prospectus supplement or free writing prospectus that we authorize from time to time, or that are incorporated by reference into this prospectus supplement or the accompanying prospectus in connection with this offering. If any of these risks actually occur, our business, financial condition, results of operations or cash flow could be seriously harmed. This could cause the trading price of our common stock to decline, resulting in a loss of all or part of your investment. Please also read carefully the section below entitled "Cautionary Note Regarding Forward-Looking Statements."*

### **Risks Related to this Offering**

***Sales of our common stock in this offering, or the perception that such sales may occur, could cause the market price of our common stock to fall.***

We may issue and sell shares of our common stock for aggregate gross proceeds of up to \$150 million from time to time in connection with this offering. The issuance and sale from time to time of these new shares of common stock, or our ability to issue these new shares of common stock in this offering could have the effect of depressing the market price of our common stock.

***Our management will have broad discretion over the use of the net proceeds from this offering, you may not agree with how we use the proceeds, and the proceeds may not be invested successfully.***

Our management will have broad discretion as to the use of the net proceeds from any offering by us and could use them for purposes other than those contemplated at the time of this offering. Accordingly, you will be relying on the judgment of our management with regard to the use of these net proceeds, and you will not have the opportunity, as part of your investment decision, to assess whether the proceeds are being used appropriately. It is possible that the proceeds will be invested in a way that does not yield a favorable, or any, return for Abeona.

***You may experience immediate and substantial dilution in the book value per share of the common stock you purchase in the offering.***

The offering price per share in this offering may exceed the pro forma net tangible book value per share of our common stock outstanding prior to this offering. Assuming that an aggregate of 10,948,905 shares of our common stock are sold at a price of \$13.70 per share, the last reported sale price of our common stock on the Nasdaq Capital Market, for aggregate gross proceeds of up to approximately \$150 million, and after deducting commissions and estimated aggregate offering expenses payable by us, you will experience immediate dilution of \$9.11 per share, representing the difference between our pro forma as adjusted net tangible book value per share as of June 30, 2018 after giving effect to this offering and the assumed offering price. The exercise of outstanding stock options will result in further dilution of your investment. See the section below entitled "Dilution" for a more detailed illustration of the dilution you would incur if you participate in this offering.

***We will require additional capital funding, the receipt of which may impair the value of our common stock.***

Our future capital requirements depend on many factors, including our research, development, sales and marketing activities. We will need to raise additional capital through public or private equity or debt offerings or through arrangements with strategic partners or other sources in order to continue to develop our drug candidates. There can be no assurance that additional capital will be available when needed or on terms satisfactory to us, if at all. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution and the new equity securities may have greater rights, preferences or privileges than our existing common stock.

## CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus supplement, the accompanying prospectus and the other documents we have filed with the SEC that are incorporated herein by reference contain forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans, objectives of management or other financial items are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “seek,” “intend,” “may,” “plan,” “predict,” “project,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus supplement, particularly as set forth and incorporated by reference in the “Risk Factors” section above, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make. You should read this prospectus supplement, the accompanying prospectus and the documents that we incorporate by reference in this prospectus supplement completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, except as otherwise required by law. We advise you, however, to consult any further disclosures we make on related subjects in our future annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K we file with or furnish to the SEC.

## USE OF PROCEEDS

We intend to use the net proceeds, if any, from this offering for working capital and general corporate purposes, including without limitation development of our product candidates and general and administrative expenses. Because there is no minimum offering amount required as a condition to close this offering, the actual total public offering amount, commissions and proceeds to us, if any, are not determinable at this time. The amounts and timing of our use of the net proceeds, if any, from the sale of securities in this offering will depend on a number of factors, such as the timing and progress of trials of our clinical and pre-clinical product candidates and our development efforts, the timing and progress of any partnering efforts, technological advances and the competitive environment for our product candidates.

As of the date of this prospectus supplement, we cannot specify with certainty all of the particular uses for the net proceeds to us from this offering. Accordingly, our management will have broad discretion in the timing and application of these proceeds. Pending application of the net proceeds as described above, we may invest the net proceeds of this offering in a variety of capital preservation investments, including but not limited to short-term, interest-bearing investment grade securities, money market accounts, certificates of deposit and direct or guaranteed obligations of the U.S. government.

## DILUTION

If you invest in this offering, your ownership interest will be diluted to the extent of the difference between the public offering price per share and the as adjusted net tangible book value per share after giving effect to this offering. We calculate net tangible book value per share by dividing the net tangible book value, which is tangible assets less total liabilities, by the number of outstanding shares of our common stock. Dilution represents the difference between the portion of the amount per share paid by purchasers of shares in this offering and the as adjusted net tangible book value per share of our common stock immediately after giving effect to this offering. Our net tangible book value as of June 30, 2018 was approximately \$124.6 million, or \$2.60 per share.

After giving effect to the sale of our common stock pursuant to this prospectus supplement and accompanying prospectus in the aggregate amount of \$150 million at an assumed offering price of \$13.70 per share, the last reported sale price of our common stock on the Nasdaq Capital Market on August 16, 2018, and after deducting commissions and estimated aggregate offering expenses payable by us, our net tangible book value as of August 16, 2018 would have been \$217.7 million, or \$4.59 per share of common stock. This represents an immediate increase in the net tangible book value of \$1.99 per share to our existing stockholders and an immediate dilution in net tangible book value of \$9.11 per share to new investors. The following table illustrates this per share dilution:

Assumed offering price per share	\$ 13.70
Net tangible book value per share as of June 30, 2018	<u>\$ 2.60</u>
Increase per share attributable to new investors	\$ 1.99
As adjusted net tangible book value per share as of August 16, 2018, after giving effect to this offering	<u>\$ 4.59</u>
Dilution per share to new investors purchasing shares in this offering	<u>\$ 9.11</u>

The table above assumes for illustrative purposes that an aggregate of 10,948,905 shares of our common stock are sold pursuant to this prospectus supplement and the accompanying prospectus at a price of \$13.70 per share, the last reported sale price of our common stock on the Nasdaq Capital Market on August 16, 2018, for aggregate gross proceeds of \$150 million. 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 \$13.70 per share shown in the table above, assuming all of our common stock in the aggregate amount of \$150 million is sold at that price, would result in an adjusted net tangible book value per share after the offering of \$4.77 per share and would increase the dilution in net tangible book value per share to new investors in this offering to \$10.23 per share, after deducting commissions and estimated aggregate 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 \$13.70 per share shown in the table above, assuming all of our common stock in the aggregate amount of \$150 million is sold at that price, would result in an adjusted net tangible book value per share after the offering of \$4.41 per share and would decrease the dilution in net tangible book value per share to new investors in this offering to \$8.59 per share, after deducting commissions and estimated aggregate offering expenses payable by us.

The foregoing table and discussion is based on 47,943,285 shares of common stock outstanding as of August 16, 2018.

## PLAN OF DISTRIBUTION

We have entered into an Open Market Sale Agreement<sup>SM</sup> (the “sales agreement”) with Jefferies LLC (the “Agent”), under which we may issue and sell shares of our common stock from time to time through the Agent acting as sales agent, subject to certain limitations, including the number of shares registered under the registration statement to which the offering relates. Sales of our common stock, if any, under this prospectus supplement and the accompanying prospectus may be made in sales deemed to be “at the market offerings” as defined in Rule 415 promulgated under the Securities Act, including sales made directly on or through the Nasdaq Capital Market, the existing trading market for our common stock, sales made to or through a market maker other than on an exchange or otherwise, in negotiated transactions at market prices prevailing at the time of sale or at prices related to such prevailing market prices, and/or any other method permitted by law. We may instruct the Agent not to sell our common stock if the sales cannot be effected at or above the price designated by us from time to time. We or the Agent may suspend the offering of our common stock upon notice and subject to other conditions. As an agent, the Agent will not engage in any transactions that stabilize the price of our common stock. Our common stock to which this prospectus supplement relates will be sold through only one Agent on any given day.

Each time we wish to issue and sell common stock under the sales agreement with the Agent, we will notify the Agent of the number of shares to be issued, the dates on which such sales are anticipated to be made, any minimum price below which sales may not be made and other sales parameters as we deem appropriate. Once we have so instructed the Agent, unless the Agent declines to accept the terms of the notice, the Agent has agreed to use its commercially reasonable efforts consistent with its normal trading and sales practices to sell such shares up to the amount specified on such terms. The obligations of the Agent under the sales agreement to sell our common stock are subject to a number of conditions that we must meet.

We will pay the Agent commissions for its services in acting as agent in the sale of our common stock. The Agent will be entitled to compensation at a commission rate equal to 3.0% of the gross sales price per share sold. Because there is no minimum offering amount required as a condition to closing this offering, the actual total public offering amount, commissions and proceeds to us, if any, are not determinable at this time. In addition, we have agreed to reimburse the Agent for fees and disbursements related to its legal counsel in an amount not to exceed \$50,000 in the aggregate.

We estimate that the total expenses for the offering, excluding compensation payable to the Agent under the terms of the sales agreement, will be approximately \$165,000.

Settlement for sales of our common stock will occur on the second business day following the date on which any sales are made, or on some other date that is agreed upon by us and the Agent in connection with a particular transaction, 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 sale of the common stock on our behalf, the Agent may, and will with respect to sales effected in an “at the market offering,” be deemed to be an “underwriter” within the meaning of the Securities Act, and the compensation of the Agent may be deemed to be underwriting commissions or discounts. We have agreed to provide indemnification and contribution to the Agent against certain civil liabilities, including liabilities under the Securities Act.

The offering pursuant to the sales agreement will terminate upon the earlier of (i) the issuance and sale of all shares of our common stock subject to the sales agreement, or (ii) the termination of such sales agreement as permitted therein.

The Agent and its affiliates may in the future provide various investment banking and other financial services for us and our affiliates, for which services they may in the future receive customary fees. To the extent required by Regulation M, the Agent will not engage in any market making activities involving our common stock while the offering is ongoing under this prospectus supplement.

## **LEGAL MATTERS**

The validity of the common stock offered hereby will be passed upon for us by Morgan, Lewis & Bockius LLP, Boston, Massachusetts. Covington & Burling LLP, New York, New York, is counsel to the Agent in connection with this offering.

## **EXPERTS**

The consolidated financial statements, incorporated by reference from the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2017, have been audited by Whitley Penn LLP, an independent registered public accounting firm, as stated in their report, which is incorporated herein by reference. Such financial statements have been so incorporated in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

**WHERE YOU CAN FIND MORE INFORMATION; INCORPORATION BY REFERENCE**

We file annual, quarterly and current reports, proxy statements and other information with the SEC. You may read and copy information filed by us with the SEC at the SEC's public reference section, 100 F Street, N.E., Washington, D.C. 20549. Information regarding the operation of the public reference section can be obtained by calling 1-800-SEC-0330. The SEC also maintains an Internet site at <http://www.sec.gov> that contains reports, statements and other information about issuers, such as us, who file electronically with the SEC. We maintain an Internet site at [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com). However, the information on our Internet sites is not incorporated by reference in this prospectus supplement and the accompanying prospectus and you should not consider it a part of this prospectus supplement or the accompanying prospectus.

The SEC allows us to "incorporate by reference" into this prospectus supplement the information in other documents that we file with it. This means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be a part of this prospectus supplement, and information in documents that we file later with the SEC will automatically update and supersede information contained in documents filed earlier with the SEC or contained in this prospectus supplement. We incorporate by reference in this prospectus supplement the documents listed below and any future filings that we may make with the SEC under Sections 13(a), 13(c), 14, or 15(d) of the Exchange Act prior to the termination of the offering under this prospectus supplement; provided, however, that we are not incorporating, in each case, any documents or information deemed to have been furnished and not filed in accordance with SEC rules:

- Our Annual Report on Form 10-K for the year ended December 31, 2017 (filed on March 16, 2018) (including the information in Part III incorporated by reference from the Company's Definitive Proxy Statement on Schedule 14A, filed on April 10, 2018);
- Our Quarterly Reports on Form 10-Q for the quarterly periods ended March 31, 2018 (filed on May 10, 2018) and June 30, 2018 (filed on August 9, 2018);
- Our Current Reports on Form 8-K filed on February 9, 2018, April 2, 2018 (only as to the items that are filed and not furnished), May 11, 2018 (as amended on May 14, 2018), July 30, 2018 and August 9, 2018; and
- The description of our common stock, par value \$0.01 per share contained in our Registration Statement on Form 8-A, dated and filed with the SEC on November 4, 2014, and any amendment or report filed with the SEC for the purpose of updating the description.

All reports and other documents we subsequently file pursuant to Section 13(a), 13(c), 14 or 15(d) of the Exchange Act prior to the termination of this offering, including all such documents we may file with the SEC after the date of the initial registration statement, but excluding any information furnished to, rather than filed with, the SEC, will also be incorporated by reference into this prospectus supplement and deemed to be part of this prospectus supplement from the date of the filing of such reports and documents.

You may obtain a copy of any or all of the documents referred to above which may have been or may be incorporated by reference into this prospectus supplement, except for exhibits to those documents (unless the exhibits are specifically incorporated by reference into those documents) at no cost to you by writing or telephoning us at the following address: Investor Relations, Abeona Therapeutics Inc., 1330 Avenue of the Americas, 33<sup>rd</sup> Floor, New York, NY 10019, telephone (216) 403-4820.

PROSPECTUS

\$500,000,000

**ABEONA THERAPEUTICS INC.**

**Common Stock**  
**Preferred Stock**  
**Warrants**  
**Debt Securities**

We may offer or sell to the public from time to time in one or more series or issuances:

- shares of our common stock;
- shares of preferred stock;
- warrants to purchase shares of our common stock, preferred stock and/or debt securities;
- debt securities consisting of debentures, notes or other evidences of indebtedness; or
- any combination of these securities.

This prospectus provides a general description of the securities that we may offer. Each time that securities are sold under this prospectus, we will provide specific terms of the securities offered in a supplement to this prospectus. The prospectus supplement may also add, update or change information contained in this prospectus. This prospectus may not be used to consummate a sale of securities unless accompanied by the applicable prospectus supplement. You should read both this prospectus and the applicable prospectus supplement together with additional information described under the heading “Where You Can Find More Information” before you make your investment decision.

Securities sold under this prospectus shall be sold directly to purchasers or through agents on our behalf or through underwriters or dealers as designated from time to time. If any agents or underwriters are involved in the sale of any of these securities, the applicable prospectus supplement will provide the names of the agents or underwriters and any applicable fees, commissions or discounts.

Our common stock is listed on the Nasdaq Capital Market under the symbol “ABEO.” On May 10, 2018, the closing price of our common stock was \$18.85.

As of May 11, 2018, the aggregate market value of our outstanding common stock held by non-affiliates was approximately \$520,690,000, based on 47,305,285 shares of outstanding common stock, of which approximately 27,622,787 shares are held by non-affiliates, and a per share price of \$18.85 based on the closing sale price of our common stock on May 10, 2018.

In addition to the securities covered under this prospectus, our securities are currently also subject to ongoing public distribution pursuant to a prospectus covering the resale of an aggregate of 1,925,000 shares of common stock pursuant to the registration statement on Form S-3 Registration No. 333-204179.

The mailing address of our principal executive offices is 1330 Avenue of the Americas, 33<sup>rd</sup> Floor, New York, NY 10019. Our telephone number is (646) 813-4705.

Investing in our securities involves certain risks. Before investing, you should read the risk factors on page [6](#) of this prospectus, included in our periodic reports, in prospectus supplements and in other information filed by us with the Securities and Exchange Commission.

This prospectus may not be used to offer or sell any securities unless accompanied by a prospectus supplement.

**Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.**

We may sell these securities on a continuous or delayed basis directly, through agents, dealers or underwriters as designated from time to time, or through a combination of these methods. We reserve the sole right to accept, and together with any agents, dealers and underwriters, reserve the right to reject, in whole or in part, any proposed purchase of securities. If any agents, dealers or underwriters are involved in the sale of any securities, the applicable prospectus supplement will set forth any applicable commissions or discounts. Our net proceeds from the sale of securities also will be set forth in the applicable prospectus supplement.

The date of this prospectus is June 7, 2018.

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## ABOUT THIS PROSPECTUS

This prospectus is part of a “shelf” registration statement. Under this process, we may sell, at any time and from time to time, in one or more offerings, any combination of the securities described in this prospectus. The exhibits to our Registration Statement contain the full text of certain contracts and other important documents we have summarized in this prospectus. Since these summaries may not contain all the information that you may find important in deciding whether to purchase the securities we offer, you should review the full text of these documents. The Registration Statement and the exhibits can be obtained from the Securities and Exchange Commission (“SEC”) as indicated under the heading “Where You Can Find More Information.”

This prospectus only provides you with a general description of the securities we may offer. Each time we sell securities, we will provide a prospectus supplement that contains specific information about the terms of those securities and the terms of that offering. The prospectus supplement may also add, update or change information contained in this prospectus. You should read both this prospectus and any prospectus supplement together with the additional information described below under the heading “Where You Can Find More Information.”

We have not authorized any dealer, agent or other person to give any information or to make any representation other than those contained or incorporated by reference into this prospectus and any accompanying prospectus supplement. You must not rely upon any information or representation not contained or incorporated by reference into this prospectus or an accompanying prospectus supplement. This prospectus and the accompanying prospectus supplement, if any, do not constitute an offer to sell or the solicitation of an offer to buy any securities other than the registered securities to which they relate, nor do this prospectus and the accompanying prospectus supplement, if any, constitute an offer to sell or the solicitation of an offer to buy securities in any jurisdiction to any person to whom it is unlawful to make such offer or solicitation in such jurisdiction. You should not assume that the information contained in this prospectus and the accompanying prospectus supplement, if any, is accurate on any date subsequent to the date set forth on the front of the document or that any information we have incorporated by reference is correct on any date subsequent to the date of the document incorporated by reference, even though this prospectus and any accompanying prospectus supplement is delivered or securities are sold on a later date.

References in this prospectus to the terms “the Company,” “Abeona,” “we,” “our” and “us” or other similar terms mean Abeona Therapeutics Inc., unless we state otherwise or the context indicates otherwise.

## THE COMPANY

### Overview

Abeona Therapeutics Inc. (together with our subsidiaries, “we,” “our,” “Abeona” or the “Company”) is a Delaware corporation. We are a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Our lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV NAGLU), an AAV based gene therapy for Sanfilippo syndrome type B (MPS IIIB). We are also developing ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition we are developing a proprietary vector platform, AIM™, for next generation product candidates. Our principal executive office is located at 1330 Avenue of the Americas, 33<sup>rd</sup> Floor, New York, NY 10019. Our website address is [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com). We do not incorporate by reference into this prospectus the information on our website, and you should not consider it as part of this prospectus.

### Recent Developments

On April 23, 2018, we announced that the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to ABO-102 our AAV-mediated gene therapy for the treatment of Sanfilippo syndrome Type A (MPS IIIA).

On April 20, 2018, we announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products has granted Orphan Drug designation (EMA/OD/013/18) for our gene therapy program ABO-202 for the treatment of subjects with neuronal ceroid lipofuscinosis, also known as Batten Disease, a fatal lysosomal storage disease that primarily affects the nervous system in children.

On March 29, 2018, F. Carsten Thiel, Ph.D. was appointed by our Board of Directors as Chief Executive Officer. Timothy J. Miller, Ph.D. will remain President and has assumed the position of Chief Scientific Officer in charge of our clinical and preclinical research programs. Dr. Thiel most recently served as the Executive Vice President and Chief Commercial Officer of Alexion Pharmaceuticals, Inc. where he led global commercial operations bringing innovative and life-transformative therapies for rare diseases to patients.

On March 15, 2018, we announced that the FDA has granted Rare Pediatric Disease Designation for the ABO-202 program (AAV-CLN1), an AAV-based gene therapy for the treatment of CLN1 disease (infantile and late infantile onset Batten disease).

### Product Development Strategy

Abeona is focused on developing and delivering gene therapy products for severe and life-threatening rare diseases. A rare disease is one that affects fewer than 200,000 people in the U.S. There are nearly 7,000 rare diseases, which may involve chronic illness, disability, and often, premature death. More than 25 million Americans and 30 million Europeans have a severe, life-threatening rare disease. While rare diseases can affect any age group, about 50% of people affected are children (15 million) and rare diseases account for 35% of deaths in the first year of life. These rare diseases are often poorly diagnosed, very complex, and have no treatment or not very effective treatment. Over 95% of rare diseases do not have a single FDA or EMA approved drug treatment, though most rare diseases are often caused by changes in genes. Approximately 80% of rare diseases are genetic in origin and can present at any stage of life. We believe emerging insights in genetics and advances in biotechnology, as well as new approaches and collaboration between researchers, industry, regulators and patient groups, provide significant opportunities to develop breakthrough treatments for rare diseases.

### Developing Next Generation Gene Therapy

Gene therapy is the use of DNA as a potential therapy to treat a disease. In many disorders, particularly genetic diseases caused by a single genetic defect, gene therapy aims to treat a disease by

delivering the correct copy of DNA into a patient's cells. The healthy, functional copy of the therapeutic gene then helps the cell function correctly. In gene therapy, DNA that encodes a therapeutic protein is packaged within a "vector," often a "naked" virus, which is used to transfer the DNA to the inside of cells within the body. Gene therapy can be delivered by a direct injection, either intravenously (IV) or directly into a specific tissue in the body, where it is taken up by individual cells. Once inside cells, the correct DNA is expressed by the cell machinery, resulting in the production of missing or defective protein, which in turn is used to treat the patient's underlying disease and can provide long-term benefit.

Abeona is developing next-generation AAV gene therapies. Viruses such as AAV are utilized because they have evolved a way of encapsulating and delivering one or more genes of the size needed for clinical application, and can be purified in large quantities at high concentration. Unlike AAV vectors found in nature, the AAV vectors used by Abeona have been genetically-modified such that they do not replicate. Although the preclinical studies in animal models of disease demonstrate the promising impact of AAV-mediated gene expression to affected tissues such as the heart, liver and muscle, our programs use a specific virus that is capable of delivering therapeutic DNA across the blood brain barrier and into the central nervous system (CNS) and the somatic system (body), making them attractive for addressing lysosomal storage diseases which have severe CNS manifestations of the disease.

Lysosomal storage diseases (LSDs) are a group of rare inborn errors of metabolism resulting from deficiency in normal lysosomal function. These diseases are characterized by progressive accumulation of storage material within the lysosomes of affected cells, ultimately leading to cellular dysfunction. Multiple tissues ranging from musculoskeletal and visceral to tissues of the CNS are typically involved in disease pathology. Since the advent of enzyme replacement therapy (ERT) to manage some LSDs, general clinical outcomes have significantly improved; however, treatment with infused protein is lifelong and continued disease progression is still evident in patients. Thus, AAV-based gene therapy may provide a viable alternative or adjunctive therapy to current management strategies for LSDs.

Our initial programs are focused on LSDs such as Mucopolysaccharidosis (MPS) III A and IIIB. MPSIII, also known as Sanfilippo syndromes type A and type B, is a progressive neuromuscular disease with profound CNS involvement. Our lead product candidates, ABO-101 and ABO-102, have been developed to replace the damaged, malfunctioning enzymes within target cells with the normal, functioning version. ABO-201 is a similar product, using an AAV to deliver the correct lysosomal gene that is defective in juvenile neuronal ceroid lipofuscinosis. Delivered via a single injection, these drugs are only given once to a patient.

***ABO-101 for MPS III B and ABO-102 for MPS III A (Sanfilippo syndrome)***

MPS III (Sanfilippo syndrome) is a group of four inherited genetic diseases, described as type A, B, C or D, which cause enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (sugars) in body tissues. MPS III is a lysosomal storage disease, a group of rare inborn errors of metabolism resulting from deficiency in normal lysosomal function. The incidence of MPS III (all four types combined) is estimated to be 1 in 70,000 births.

Mucopolysaccharides are long chains of sugar molecules used in the building of connective tissues in the body. There is a continuous process in the body of replacing used materials and breaking them down for disposal. Children with MPS III are missing an enzyme which is essential in breaking down used mucopolysaccharides. The partially broken down mucopolysaccharides remain stored in cells in the body causing progressive damage. Babies may show little sign of the disease, but as more and more cells become damaged, symptoms start to appear.

In MPS III, the predominant symptoms occur due to accumulation within the CNS, including the brain and spinal cord, resulting in cognitive decline, motor dysfunction, and eventual death. To date, there is no cure for MPS III and treatments are largely supportive.

Abeona is developing next-generation AAV-based gene therapies for MPS III, which involves a one-time delivery of a normal copy of the defective gene to cells of the CNS with the aim of reversing the effects of the genetic errors that cause the disease.

After a single dose in MPS III preclinical models, ABO-101 and ABO-102 induced cells in the CNS and peripheral organs to produce the missing enzymes which helped repair the damage caused to the cells. Preclinical *in vivo* efficacy studies in MPS III have demonstrated functional benefits that remain for months after treatment. A single dose of ABO-101 or ABO-102 significantly restored normal cell and organ function, corrected cognitive defects that remained months after drug administration, increased neuromuscular control and increased the lifespan of animals with MPS III over 100% one year after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with MPS III A and B. In addition, safety studies conducted in animal models of MPS III have demonstrated that delivery of ABO-101 or ABO-102 are well tolerated with minimal side effects.

***EB-101 for the Treatment of Recessive Dystrophic Epidermolysis Bullosa (“RDEB”) and EB-201 for the Correction of Gene Mutations in Skin Cells (Keratinocytes)***

EB-101 (LZRSE-Col7A1 Engineered Autologous Epidermal Sheets (LEAES)), is an ex vivo gene therapy for the treatment of RDEB. EB-201 (AAVDJ-Col7A1) is a pre-clinical candidate targeting a novel, AAV-mediated gene editing and delivery approach to correct gene mutations in skin cells for patients with RDEB. We entered into an agreement (the “EB Agreement”) with EB Research Partnership (“EBRP”) and Epidermolysis Bullosa Medical Research Foundation (“EBMRF”) to collaborate on gene therapy treatments for EB. The EB Agreement became effective August 3, 2016, on the execution of two licensing agreements with The Board of Trustees of Leland Stanford Junior University (“Stanford”) described below.

We entered into a license with Stanford effective August 3, 2016 for the EB-101 (LZRSE-Col7A1 Engineered Autologous Epidermal Sheets (LEAES)) technology, and we have performed certain preclinical development work and are performing clinical trials of a gene therapy treatment for EB based upon such in-licensed technology.

We also entered into a license with Stanford effective August 3, 2016 for the EB-201 (AAV DJ COL7A1) technology, and we plan to perform preclinical development and clinical trials of a gene therapy treatment for EB based upon such in-licensed technology.

***ABO-201 for juvenile Batten disease (or Juvenile Neuronal Ceroid Lipofuscinoses) (“JNCL”) and ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (or Infantile Neuronal Ceroid Lipofuscinoses) (“INCL”)***

ABO-201 (AAV CLN3) is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective CLN3 gene to cells of the CNS with the aim of reversing the effects of the genetic errors that cause JNCL. JNCL is a rare, fatal, autosomal recessive (inherited) disorder of the nervous system that typically begins in children between 4 and 8 years of age. Often the first noticeable sign of JNCL is vision impairment, which tends to progress rapidly and eventually result in blindness. As the disease progresses, children experience loss of previously acquired skills (developmental regression). This regression usually begins with the loss of the ability to speak in complete sentences. Children then lose motor skills, such as the ability to walk or sit. They also develop movement abnormalities that include rigidity or stiffness, slow or diminished movements (hypokinesia), and stooped posture. Beginning in mid to late childhood, affected children may have recurrent seizures (epilepsy), heart problems, behavioral problems, and difficulty sleeping. Life expectancy is greatly reduced. Most people with juvenile Batten disease live into their twenties or thirties. As yet, no specific treatment is known that can halt or reverse the symptoms of JNCL.

JNCL is the most common form of a group of disorders known as neuronal ceroid lipofuscinoses (NCLs). Collectively, all forms of NCL affect an estimated 2 to 4 in 100,000 live births in the United States. NCLs are more common in Finland, where approximately 1 in 12,500 individuals are affected, as well as Sweden, other parts of northern Europe, and Newfoundland, Canada.

Most cases of JNCL are caused by mutations in the CLN3 gene, which is the focus of our AAV-based gene therapy approach. These mutations disrupt the function of cellular structures called lysosomes. Lysosomes are compartments in the cell that normally digest and recycle different types of molecules. Lysosome malfunction leads to a buildup of fatty substances called lipopigments and proteins within these

cell structures. These accumulations occur in cells throughout the body, but neurons in the brain seem to be particularly vulnerable to damage. The progressive death of cells, especially in the brain, leads to vision loss, seizures, and intellectual decline in children with JNCL.

ABO-202 (AAV9 CLN1) is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective CLN1 gene to cells of the central nervous system with the aim of reversing the effects of the genetic errors that cause an infantile form of Batten disease (also known as infantile neuronal ceroid lipofuscinosis).

***ABO-301 for Fanconi Anemia (“FA”) and ABO-302 for rare blood diseases using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases***

ABO-301 (AAV-FANCC) is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective gene to cells of the hematopoietic or blood system with the aim of reversing the effects of the genetic errors that cause FA. FA is a rare (1 in 160,000) pediatric, autosomal recessive (inherited) disease characterized by multiple physical abnormalities, organ defects, bone marrow failure, and a higher than normal risk of cancer. The average lifespan for people with FA is 20 to 30 years.

The major function of bone marrow is to produce new blood cells. In FA, a DNA mutation renders the FANCC gene nonfunctional. Loss of FANCC causes skeletal abnormalities and leads to bone marrow failure. FA patients also have much higher rates of hematological diseases, such as acute myeloid leukemia or tumors of the head, neck, skin, gastrointestinal system, or genital tract. The likelihood of developing one of these cancers in people with FA is between 10 and 30 percent. Aside from bone marrow transplantation, there are no specific treatments known that can halt or reverse the symptoms of FA. Repairing fibroblast cells in FA patients with a functional FANCC gene is the focus of our AAV-based gene therapy approach.

Using a novel CRISPR (clustered, regularly interspaced short palindromic repeats)-Cas9 (CRISPR associated protein 9) system, researchers used a protein-RNA complex composed of an enzyme known as Cas9 bound to a guide RNA molecule that has been designed to recognize a particular DNA sequence. The RNA molecules guide the Cas9 complex to the location in the genome that requires repair. CRISPR-Cas9 uniquely enables surgically efficient knock-out, knock-down or selective editing of defective genes in the context of their natural promoters, unlocking the potential to treat both recessive and dominant forms of genetic diseases. Most importantly, this approach has the potential to allow for more precise gene modification.

**Polymer Hydrogel Technology (PHT™)**

***MuGard® (mucoadhesive oral wound rinse) approved for mucositis, stomatitis, aphthous ulcers, and traumatic ulcers***

MuGard is our marketed product for the management of oral mucositis, a frequent side-effect of cancer therapy for which there is no other established treatment. MuGard, a proprietary nanopolymer formulation, received marketing clearance from the FDA in the U.S. as well as Europe, China, Australia, New Zealand and Korea. We launched MuGard in the U.S. in 2010 and licensed MuGard for commercialization in the U.S. to AMAG Pharmaceuticals, Inc. (AMAG) in 2013. We licensed MuGard to RHEI Pharmaceuticals, N.V. for China and other Southeast Asian countries in 2010; Hanmi Pharmaceutical Co. Ltd. for South Korea in 2014; and Norgine B.V. for the European Union, Switzerland, Norway, Iceland, Lichtenstein, Australia and New Zealand in 2014.

## RISK FACTORS

An investment in our securities involves risks. We urge you to consider carefully the risks described below, and in the documents incorporated by reference into this prospectus and, if applicable, in any prospectus supplement used in connection with an offering of our securities, before making an investment decision, including those risks identified under “Item 1A. Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2017, which is incorporated by reference into this prospectus and which may be amended, supplemented or superseded from time to time by other reports that we subsequently file with the SEC. Additional risks, including those that relate to any particular securities we offer, may be included in a prospectus supplement or free writing prospectus that we authorize from time to time, or that are incorporated by reference into this prospectus or a prospectus supplement.

### *Risks related to the discovery and development of our product candidates*

**Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. At the moment, few gene therapy products have been approved in the United States and in the European Union (EU).**

We have concentrated our therapeutic product research and development efforts on our gene therapy platform, and our future success depends on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the European Medicines Agency (EMA) and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. At the moment, only one gene therapy product, UniQure’s Glybera, which received marketing authorization in the EU in 2012, has been approved in the Western world, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have evolved and may continue to change in the future. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical studies conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities’ Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical study, even if the FDA has reviewed the study and approved its initiation. Conversely, the FDA can put an investigational new drug application, or IND, on clinical hold even if the RAC has provided a favorable review. Also, before a clinical study can begin at an NIH-funded institution, that institution’s institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical study to assess the safety of the study. In addition, serious adverse events or developments in clinical trials of gene therapy product candidates conducted by others may cause the FDA or other regulatory bodies to initiate a clinical hold on our clinical trials or otherwise change the requirements for approval of any of our product candidates.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or

prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

**We may find it difficult to enroll patients in our clinical studies, which could delay or prevent clinical studies of our product candidates.**

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends on the speed at which we can recruit patients to participate in testing our product candidates. In our experience, we have experienced delays in some of our upcoming clinical studies, and we may experience similar delays in the future. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical studies for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical studies altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical studies in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the study protocol;
- number of patients in the eligible patient population;
- weight of the eligible patient population, since our gene therapies are dosed per/kg;
- amount of drug available during a particular year;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- patient referral practices of physicians;
- judgment and decisions of the clinical site investigators;
- clinical site institutional review board review and judgment; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions for which we plan to evaluate our current gene therapy product candidates are rare genetic disorders with limited patient pools from which to draw for clinical studies. The eligibility criteria of our gene therapy clinical studies will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly.

Certain of our current product candidates are being developed to treat rare conditions. We plan to seek initial marketing approval in the United States and the European Union, and may have clinical studies in Australia, South America and Japan. We may not be able to initiate or continue clinical studies if we cannot

enroll a sufficient number of eligible patients to participate in the clinical studies required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for the conduct of clinical studies;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Because the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials at such clinical trial sites. Moreover, because our product candidates represent a departure from more commonly used methods for rare disease and/or cancer treatment, potential patients and their doctors may be inclined to use conventional therapies, such as chemotherapy and hematopoietic cell transplantation, rather than enroll patients in any future clinical trial.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical studies as planned, we may need to delay, limit or terminate ongoing or planned clinical studies, any of which would have an adverse effect on our business. Even if we are able to enroll a sufficient number of patients in our clinical trials, delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

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

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

- delays in reaching a consensus with regulatory agencies on study design;
- delays in obtaining required Institutional Review Board, or IRB, or Institutional Ethics Committee approval at each clinical study site;
- delays in recruiting suitable patients to participate in our clinical studies;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical study operations or study sites or due to unforeseen safety issues;
- failure by our CROs, other third parties or us to adhere to clinical study requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory requirements in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;

- failure to obtain sufficient drug product during manufacture to achieve target doses;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical study sites or patients dropping out of a study;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to demonstrate comparability of our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

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

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

Treatment with our gene therapy product candidates involves gene infusion, muscle and nerve studies and radiology studies, which can cause side effects or adverse events that are unrelated to our product candidate, but may still impact the success of our clinical studies. Additionally, our product candidates could potentially cause other adverse events that have not yet been predicted. The inclusion of critically ill patients in our clinical studies may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using. As described above, any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

**We have not completed any clinical studies of our current viral vectors or product candidates derived from these viral vectors or gene editing strategies. Success in early clinical studies may not be indicative of results obtained in later studies.**

We have not initiated evaluation in human clinical studies of our current viral vectors and our product candidates, and we may experience unexpected results in the future. Earlier gene therapy clinical studies, which we believe serve as proof-of-concept for our product candidates, utilized lentiviral or adenoviral vectors similar to ours. However, these studies should not be relied upon as evidence that our future clinical studies will succeed. Study designs and results from previous studies are not necessarily predictive of our

future clinical study designs or results, and initial results may not be confirmed upon full analysis of the complete study data. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies.

There is a high failure rate for drugs and biologics proceeding through clinical studies. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical studies even after achieving promising results in earlier stage clinical studies. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

**Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our gene therapies and our product candidates or adversely affect our ability to conduct our business or obtain further marketing approvals for our gene therapies and marketing approvals for our product candidates.**

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing symptomatic treatments they are already familiar with and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize murine gamma-retroviral vectors, our product candidates use a viral delivery system. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. Although none of our current product candidates utilize the gamma-retroviruses used in the 2003 studies, our product candidates do use a viral vector delivery system. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical studies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

Adverse events in our clinical trials or those conducted by other parties, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. If any such adverse events occur, commercialization of AAV-based gene therapies or further advancement of our clinical trials could be halted or delayed, which would have a material adverse effect on our business and operations.

**In previous clinical studies involving viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of leukemia due to vector-related insertional oncogenesis. Other clinical trials have demonstrated significant immune responses or adverse events related to over-production of the transgene after injection of the viral vector. If our vectors demonstrate a similar effect, we may be required to halt or delay further clinical development of our product candidates.**

A significant risk in any gene therapy product based on viral vectors is that the vector will insert in or near cancer-causing oncogenes leading to uncontrolled clonal proliferation of mature cancer cells in the patient. For example, in 2003, 20 subjects treated for X-linked severe combined immunodeficiency in two gene therapy studies using a murine, or mouse-derived, gamma-retroviral vector showed correction of the

disease, but the studies were terminated after five subjects developed leukemia (four of whom were subsequently cured). The cause of these adverse events was shown to be insertional oncogenesis, which is the process whereby the corrected gene inserts in or near a gene that is important in a critical cellular process like growth or division, and this insertion results in the development of a cancer (often leukemia). Using molecular diagnostic techniques, it was determined that clones from these subjects showed retrovirus insertion in proximity to the promoter of the LMO2 proto-oncogene. Earlier generation retroviruses like the one used in these two studies have been shown to preferentially integrate in regulatory regions of genes that control cell growth.

These well-publicized adverse events led to the development of new viral vectors, such as lentiviral and adeno-associated virus (AAV) vectors, with improved safety profiles and also the requirement of enhanced safety monitoring in gene therapy clinical trials, including periodic analyses of the therapy's genetic insertion sites. In published studies, lentiviral vectors have demonstrated an improved safety profile over gamma-retroviral vectors, with no disclosed events of gene therapy-related adverse events, which we believe is due to a number of factors including the tendency of these vectors to integrate within genes rather than in areas that control gene expression, as well as their lack of strong viral enhancers.

Notwithstanding the historical data regarding the potential safety improvements of lentiviral vectors, the risk of insertional oncogenesis remains a significant concern for gene therapy and we cannot assure that it will not occur in any of our ongoing or planned clinical studies. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. The FDA has stated that lentiviral vectors possess characteristics that may pose high risks of delayed adverse events. If any such adverse events occur, further advancement of our clinical studies could be halted or delayed, which would have a material adverse effect on our business and operations.

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

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

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

Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved biologics license application, or BLA, is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with

good manufacturing practices, or GMP, and adherence to commitments made in the BLA. If we or a regulatory agency discovers previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical studies;
- refuse to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

***Risks related to our reliance on third parties***

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

We do not expect to independently conduct all aspects of our vector production, product manufacturing, research and preclinical and clinical testing, monitoring and data-analysis. We currently rely, and expect to continue to rely, on third parties with respect to these items. In some cases these third parties are academic, research or similar institutions that may not apply the same quality control protocols utilized in certain commercial settings.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical studies are conducted in accordance with the study plan and protocols.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies required to support future IND and BLA submissions and approval of our product candidates.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities.

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

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;

- the risk that these activities are not conducted in accordance with our study plans and protocols;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier, and
- FDA inspection of company manufacturing facilities that result in a clinical hold and/or revision of company manufacturing practices.

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

**Gene- and cell-based therapies rely on the availability of reagents, specialized equipment, and other specialty materials, which may not be available to us on acceptable terms or at all. For some of these reagents, equipment, and materials, we rely or may rely on sole source vendors or a limited number of vendors, which could impair our ability to manufacture and supply our products.**

Manufacturing our product candidates will require many reagents, which are substances used in our manufacturing processes to bring about chemical or biological reactions, and other specialty materials and equipment, some of which are manufactured or supplied by small companies with limited resources and experience to support commercial biologics production. We currently depend on a limited number of vendors for certain materials and equipment used in the manufacture of our product candidates. Some of these suppliers may not have the capacity to support commercial products manufactured under Current Good Manufacturing Practices (“CGMP”) by biopharmaceutical firms or may otherwise be ill-equipped to support our needs. We also do not have supply contracts with many of these suppliers and may not be able to obtain supply contracts with them on acceptable terms or at all. Accordingly, we may experience delays in receiving key materials and equipment to support clinical or commercial manufacturing.

For some of these reagents, equipment, and materials, we rely and may in the future rely on sole source vendors or a limited number of vendors. An inability to continue to source product from any of these suppliers, which could be due to regulatory actions or requirements affecting the supplier, adverse financial or other strategic developments experienced by a supplier, labor disputes or shortages, unexpected demands, or quality issues, could adversely affect our ability to satisfy demand for our product candidates, which could adversely and materially affect our product sales and operating results or our ability to conduct clinical trials, either of which could significantly harm our business.

As we continue to develop and scale our manufacturing process, we expect that we will need to obtain rights to and supplies of certain materials and equipment to be used as part of that process. We may not be able to obtain rights to such materials on commercially reasonable terms, or at all, and if we are unable to alter our process in a commercially viable manner to avoid the use of such materials or find a suitable substitute, it would have a material adverse effect on our business. Even if we are able to alter our process so as to use other materials or equipment, such a change may lead to a delay in our clinical development and/or commercialization plans. If such a change occurs for a product candidate that is already in clinical testing, the change may require us to perform both *ex vivo* comparability studies and to collect additional data from patients prior to undertaking more advanced clinical trials.

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

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

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Some components of a finished therapeutic product approved for commercial sale or used in late-stage clinical studies must be manufactured in accordance with good manufacturing practice, or GMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or marketing authorization application, or MAA, on a timely basis and where required, must adhere to the FDA's or other regulator's good laboratory practices, or GLP, and GMP regulations enforced by the FDA or other regulator through facilities inspection programs. Some of our contract manufacturers have not produced a commercially-approved product and therefore have not obtained the requisite FDA or other regulatory approvals to do so. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA or other regulatory approval of the products will not be granted.

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

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

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. The number of manufacturers with the necessary manufacturing capabilities is limited. In addition, an alternative manufacturer would need to be qualified through a BLA supplement or similar regulatory submission which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

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

**We expect to rely on third parties to conduct, supervise and monitor our clinical studies, and if these third parties perform in an unsatisfactory manner, it may harm our business.**

We expect to rely on CROs and clinical study sites to ensure our clinical studies are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with the FDA's good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical study participants are protected. The FDA enforces these GCPs through periodic inspections of study sponsors, principal investigators and clinical study sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our future clinical studies may be deemed unreliable and the FDA may require us to perform additional clinical studies before approving any marketing applications. Upon inspection, the FDA may determine that our clinical studies did not comply with GCPs. In addition, our future clinical studies will require a sufficient number of test subjects to evaluate the safety and efficacy of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical studies, which would delay the regulatory approval process.

Employees of our CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs, which must be conducted in accordance with GCPs and good laboratory practices, or GLPs, respectively. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies or other drug development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical studies may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

We also expect to rely on other third parties to store and distribute our vectors and products for any clinical studies that we may conduct. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential product revenue.

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

Because we rely on third parties to manufacture our vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through

breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

### ***Risks Relating to our Business and Industry***

#### **We have experienced a history of losses, we expect to incur future losses and we may be unable to obtain necessary additional capital to fund operations in the future.**

We have recorded minimal revenue to date and have incurred an accumulated deficit of approximately \$359.8 million through December 31, 2017 and \$332.5 million through December 31, 2016. Net loss allocable to common stockholders for the year ended December 31, 2017 was \$27.3 million and the net loss for the year ended December 31, 2016 was \$21.9 million. Our losses have resulted principally from costs incurred in research and development activities related to our efforts to develop clinical drug candidates, from losses due to derivatives and from the associated administrative costs. We expect to incur additional operating losses over the next several years. We also expect cumulative losses to increase if we expand research and development efforts and preclinical and clinical trials.

We require substantial capital for our development programs and operating expenses, to pursue regulatory clearances and to prosecute and defend our intellectual property rights. We will need to raise substantial additional capital to support our ongoing and planned operations.

If we raise additional funds by issuing equity securities, further dilution to existing stockholders will result and future investors may be granted rights superior to those of existing stockholders. If adequate funds are not available to us through additional equity offerings, we may be required to delay, reduce the scope of or eliminate one or more of our research and development programs or to obtain funds by entering into arrangements with collaborative partners or others that require us to issue additional equity securities or to relinquish rights to certain technologies or drug candidates that we would not otherwise issue or relinquish in order to continue independent operations.

#### **We do not have significant operating revenue and may never attain profitability.**

To date, we have funded our operations primarily through private sales of common stock, preferred stock and convertible notes as well as public offerings of our common stock. Contract research payments and licensing fees from corporate alliances and mergers have also provided funding for our operations. Our ability to achieve significant revenue or profitability depends upon our licensees' ability to successfully market MuGard in North America, Europe, Australia, New Zealand, Korea and China or to complete the development of our drug candidates, to develop and obtain patent protection and regulatory approvals for our drug candidates and to manufacture and commercialize the resulting drugs. We are not expecting any significant revenues in the short-term from our products or product candidates. Furthermore, we may not be able to ever successfully identify, develop, commercialize, patent, manufacture, obtain required regulatory approvals and market any additional products. Moreover, even if we do identify, develop, commercialize, patent, manufacture, and obtain required regulatory approvals to market additional products, we may not generate revenues or royalties from commercial sales of these products for a significant number of years, if at all. Therefore, our proposed operations are subject to all the risks inherent in the establishment of a new business enterprise. In the next few years, our revenues may be limited to minimal product sales and royalties, and any amounts that we receive under strategic partnerships and research or drug development collaborations that we may establish and, as a result, we may be unable to achieve or maintain profitability in the future or to achieve significant revenues in order to fund our operations.

#### **We may not successfully commercialize our drug candidates.**

Our drug candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe commercially viable drugs would severely limit our ability to become profitable or to achieve significant revenues. We may be unable to successfully commercialize our drug candidates because:

- some or all of our drug candidates may be found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances;
- our drug candidates, if safe and effective, may be too difficult to develop into commercially viable drugs;
- it may be difficult to manufacture or market our drug candidates on a large scale;
- proprietary rights of third parties may preclude us from marketing our drug candidates; and
- third parties may market superior or equivalent drugs.

**The success of our research and development activities, upon which we primarily focus, is uncertain.**

Our primary focus is on our research and development activities and the commercialization of compounds covered by proprietary biopharmaceutical patents and patent applications. Research and development activities, by their nature, preclude definitive statements as to the time required and costs involved in reaching certain objectives. Actual research and development costs, therefore, could significantly exceed budgeted amounts and estimated time frames may require significant extension. Cost overruns, unanticipated regulatory delays or demands, unexpected adverse side effects or insufficient therapeutic efficacy will prevent or substantially slow our research and development effort and our business could ultimately suffer. We anticipate that we will remain principally engaged in research and development activities for an indeterminate, but substantial, period of time.

**We may be unable to successfully develop, market, or commercialize our products or our product candidates without establishing new relationships and maintaining current relationships and our ability to successfully commercialize, and market our product candidates could be limited if a number of these existing relationships are terminated.**

Our strategy for the research, development and commercialization of our potential pharmaceutical products may require us to enter into various arrangements with corporate and academic collaborators, licensors, licensees and others, in addition to our existing relationships with other parties. Specifically, we may seek to enter joint venture, sublicense or other marketing arrangements with parties that have an established marketing capability or we may choose to pursue the commercialization of such products on our own. We may, however, be unable to establish such additional collaborative arrangements, license agreements, or marketing agreements as we may deem necessary to develop, commercialize and market our potential pharmaceutical products on acceptable terms. Furthermore, if we maintain and establish arrangements or relationships with third parties, our business may depend upon the successful performance by these third parties of their responsibilities under those arrangements and relationships.

**We may be unable to successfully manufacture our products and our product candidates in clinical quantities or for commercial purposes without the assistance of contract manufacturers, which may be difficult for us to obtain and maintain.**

We have limited experience in the manufacture of pharmaceutical products in clinical quantities or for commercial purposes and we may not be able to manufacture any new pharmaceutical products that we may develop. As a result, we have established, and in the future intend to establish, arrangements with contract manufacturers to supply sufficient quantities of products to conduct clinical trials and for the manufacture, packaging, labeling and distribution of finished pharmaceutical products if any of our potential products are approved for commercialization. If we are unable to contract for a sufficient supply of our potential pharmaceutical or biopharmaceutical products on acceptable terms, our preclinical and human clinical testing schedule may be delayed, resulting in the delay of our clinical programs and submission of product candidates for regulatory approval. This may cause our business to suffer if there are delays or difficulties in establishing relationships with manufacturers to produce, package, label and distribute our finished pharmaceutical or biopharmaceutical or other medical products, if any. Moreover, US contract manufacturers that we may use must adhere to current Good Manufacturing Practices, as required by the FDA. In this regard, the FDA will not issue a pre-market approval or product and establishment licenses, where applicable, to a manufacturing facility for the products until the

manufacturing facility passes a pre-approval plant inspection. If we are unable to obtain or retain third party manufacturing on commercially acceptable terms, we may not be able to commercialize our products as planned. Our potential dependence upon third parties for the manufacture of our products may adversely affect our ability to generate profits or acceptable profit margins and our ability to develop and deliver such products on a timely and competitive basis.

**We are subject to extensive governmental regulation which increases our cost of doing business and may affect our ability to commercialize any new products that we may develop.**

The FDA and comparable agencies in foreign countries impose substantial requirements upon the introduction of pharmaceutical products through lengthy and detailed laboratory, preclinical and clinical testing procedures and other costly and time-consuming procedures to establish safety and efficacy. All of our drugs and drug candidates require receipt and maintenance of governmental approvals for commercialization. Preclinical and clinical trials and manufacturing of our drug candidates will be subject to the rigorous testing and approval processes of the FDA and corresponding foreign regulatory authorities. Satisfaction of these requirements typically takes a significant number of years and can vary substantially based upon the type, complexity and novelty of the product.

Due to the time-consuming and uncertain nature of the drug candidate development process and the governmental approval process described above, we cannot assure you when we, independently or with our collaborative partners, might submit a New Drug Application, or NDA, for FDA or other regulatory review. Further, our ability to commence and/or complete development projects will be subject to our ability to raise enough funds to pay for the development costs of these projects. Government regulation also affects the manufacturing and marketing of pharmaceutical products. Government regulations may delay marketing of our potential drugs for a considerable or indefinite period of time, impose costly procedural requirements upon our activities and furnish a competitive advantage to larger companies or companies more experienced in regulatory affairs. Delays in obtaining governmental regulatory approval could adversely affect our marketing as well as our ability to generate significant revenues from commercial sales.

Our drug candidates may not receive FDA or other regulatory approvals on a timely basis or at all. Moreover, if regulatory approval of a drug candidate is granted, such approval may impose limitations on the indicated use for which such drug may be marketed. Even if we obtain initial regulatory approvals for our drug candidates, our drugs and our manufacturing facilities would be subject to continual review and periodic inspection, and later discovery of previously unknown problems with a drug, manufacturer or facility may result in restrictions on the marketing or manufacture of such drug, including withdrawal of the drug from the market. The FDA and other regulatory authorities stringently apply regulatory standards and failure to comply with regulatory standards can, among other things, result in fines, denial or withdrawal of regulatory approvals, product recalls or seizures, operating restrictions and criminal prosecution.

**The uncertainty associated with preclinical and clinical testing may affect our ability to successfully commercialize new products.**

Before we can obtain regulatory approvals for the commercial sale of any of our potential drugs, the drug candidates will be subject to extensive preclinical and clinical trials to demonstrate their safety and efficacy in humans. Preclinical or clinical trials of future drug candidates may not demonstrate the safety and efficacy to the extent necessary to obtain regulatory approvals and our drug candidates could result in injury or death to patients in our clinical trials. In this regard, for example, adverse side effects can occur during the clinical testing of a new drug on humans which may delay ultimate FDA approval or even lead it to terminate our efforts to develop the drug for commercial use. Companies in the biotechnology industry have suffered significant setbacks in advanced clinical trials, including injury or death, even after demonstrating promising results in earlier trials. The failure to adequately demonstrate the safety and efficacy of a drug candidate under development could delay or prevent regulatory approval of the drug candidate. A delay or failure to receive regulatory approval for any of our drug candidates could prevent us from successfully commercializing such candidates and we could incur substantial additional expenses in our attempt to further develop such candidates and obtain future regulatory approval.

**We may incur substantial product liability expenses due to the use or misuse of our products for which we may be unable to obtain insurance coverage.**

Our business exposes us to potential liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical products. These risks will expand with respect to our drug candidates, if any, that receive regulatory approval for commercial sale and we may face substantial liability for damages in the event of adverse side effects, including injury or death, or product defects identified with any of our products that are used in clinical tests or marketed to the public. Product liability insurance for the biotechnology industry is generally expensive, if available at all, and as a result, we may be unable to obtain insurance coverage at acceptable costs or in a sufficient amount in the future, if at all. We may be unable to satisfy any claims for which we may be held liable as a result of the use or misuse of products which we developed, manufactured or sold and any such product liability claim could adversely affect our business, operating results or financial condition.

**Intense competition may limit our ability to successfully develop and market commercial products.**

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Our competitors in the U.S. and elsewhere are numerous and include, among others, major multinational pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions. Many of our competitors have and employ greater financial and other resources, including larger research and development, marketing and manufacturing organizations. As a result, our competitors may successfully develop technologies and drugs that are more effective or less costly than any that we are developing or which would render our technology and future products obsolete and noncompetitive.

In addition, some of our competitors have greater experience than we do in conducting preclinical and clinical trials and obtaining FDA and other regulatory approvals. Accordingly, our competitors may succeed in obtaining FDA or other regulatory approvals for drug candidates more rapidly than we can. Companies that complete clinical trials, obtain required regulatory agency approvals and commence commercial sale of their drugs before their competitors may achieve a significant competitive advantage. Drugs resulting from our research and development efforts or from our joint efforts with collaborative partners therefore may not be commercially competitive with our competitors' existing products or products under development.

**Our ability to successfully develop and commercialize our drug candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.**

Market acceptance and sales of our product candidates may depend on coverage and reimbursement policies and health care reform measures. Decisions about formulary coverage as well as levels at which government authorities and third-party payers, such as private health insurers and health maintenance organizations, reimburse patients for the price they pay for our products as well as levels at which these payors pay directly for our products, where applicable, could affect whether we are able to commercialize these products. We cannot be sure that reimbursement will be available for any of these products. Also, we cannot be sure that coverage or reimbursement amounts will not reduce the demand for, or the price of, our products. We have not commenced efforts to have our product candidates reimbursed by government or third party payors. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to commercialize our products. In recent years, officials have made numerous proposals to change the health care system in the U.S. These proposals include measures that would limit or prohibit payments for certain medical treatments or subject the pricing of drugs to government control. In addition, in many foreign countries, particularly the countries of the European Union, the pricing of prescription drugs is subject to government control. If our products are or become subject to government regulation that limits or prohibits payment for our products, or that subjects the price of our products to governmental control, we may not be able to generate revenue, attain profitability or commercialize our products.

As a result of legislative proposals and the trend towards managed health care in the U.S., third-party payors are increasingly attempting to contain health care costs by limiting both coverage and the level of reimbursement of new drugs. They may also impose strict prior authorization requirements and/or refuse to

provide any coverage of uses of approved products for medical indications other than those for which the FDA has granted market approvals. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly-approved drugs, which in turn will put pressure on the pricing of drugs.

**The market may not accept any pharmaceutical products that we develop.**

The drugs that we are attempting to develop may compete with a number of well-established drugs manufactured and marketed by major pharmaceutical companies. The degree of market acceptance of any drugs developed by us will depend on a number of factors, including the establishment and demonstration of the clinical efficacy and safety of our drug candidates, the potential advantage of our drug candidates over existing therapies and the reimbursement policies of government and third-party payers. Physicians, patients or the medical community in general may not accept or use any drugs that we may develop independently or with our collaborative partners and if they do not, our business could suffer.

**Healthcare reform measures could hinder or prevent our product candidates' commercial success.**

The U.S. government and other governments have shown significant interest in pursuing healthcare reform. Any government-adopted reform measures could adversely impact the pricing of healthcare products and services in the U.S. or internationally and the amount of reimbursement available from governmental agencies or other third party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce health care costs may adversely affect our ability to set prices for our products which we believe are fair, and our ability to generate revenues and achieve and maintain profitability.

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, that relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the U.S., new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, in March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA. This law has substantially changed the way healthcare is financed by both government health plans and private insurers, and has significantly affected the pharmaceutical industry. The PPACA contains a number of provisions that are expected to impact our business and operations in ways that may negatively affect our potential revenues in the future. For example, the PPACA imposes a non-deductible excise tax on pharmaceutical manufacturers or importers that sell branded prescription drugs to U.S. government programs which we believe will increase the cost of our products. In addition, as part of the PPACA's provisions closing a funding gap that currently exists in the Medicare Part D prescription drug program (commonly known as the "donut hole"), we will be required to provide a discount on branded prescription drugs equal to 50% of the government-negotiated price, for drugs provided to certain beneficiaries who fall within the donut hole. Similarly, PPACA increases the level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1% and requires collection of rebates for drugs paid by Medicaid managed care organizations. The PPACA also includes significant changes to the 340B drug discount program including expansion of the list of eligible covered entities that may purchase drugs under the program. At the same time, the expansion in eligibility for health insurance benefits created under PPACA is expected to increase the number of patients with insurance coverage who may receive our products. While it is too early to predict all the specific effects the PPACA or any future healthcare reform legislation will have on our business, they could have a material adverse effect on our business and financial condition.

Congress periodically adopts legislation like the PPACA and the Medicare Prescription Drug, Improvement and Modernization Act of 2003, that modifies Medicare reimbursement and coverage policies pertaining to prescription drugs. Implementation of these laws is subject to ongoing revision through

regulatory and sub regulatory policies. Congress also may consider additional changes to Medicare policies, potentially including Medicare prescription drug policies, as part of ongoing budget negotiations. While the scope of any such legislation is uncertain at this time, there can be no assurances that future legislation or regulations will not decrease the coverage and price that we may receive for our proposed products. Other third-party payors are increasingly challenging the prices charged for medical products and services. It will be time consuming and expensive for us to go through the process of seeking coverage and reimbursement from Medicare and private payors. Our proposed products may not be considered cost-effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our proposed products on a profitable basis. Further federal and state proposals and health care reforms are likely which could limit the prices that can be charged for the product candidates that we develop and may further limit our commercial opportunities. Our results of operations could be materially adversely affected by proposed healthcare reforms, by the Medicare prescription drug coverage legislation, by the possible effect of such current or future legislation on amounts that private insurers will pay and by other health care reforms that may be enacted or adopted in the future. In September 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in delays or increased costs during product development, clinical trials and regulatory review, increased costs to assure compliance with post-approval regulatory requirements, and potential restrictions on the sale and/or distribution of approved products.

**Our business could suffer if we lose the services of, or fail to attract, key personnel.**

We are highly dependent upon the efforts of our senior management, including our Executive Chairman, Principal Executive Officer, and board member, Steven H. Rouhandeh; our Chief Executive Officer, F. Carsten Thiel; our President and Chief Scientific Officer, and board member, Timothy J. Miller; our Chief Operating Officer, Jeffrey B. Davis; and our Chief Accounting Officer, Stephen B. Thompson. The loss of the services of these individuals could delay or prevent the achievement of our research, development, marketing, or product commercialization objectives. We do not have employment contracts with our other key personnel. We do not maintain any 'key-man' insurance policies on any of our key employees and we do not intend to obtain such insurance. In addition, due to the specialized scientific nature of our business, we are highly dependent upon our ability to attract and retain qualified scientific and technical personnel and consultants. In view of the stage of our development and our research and development programs, we have restricted our hiring to research scientists, consultants and a small administrative staff and we have made only limited investments in manufacturing, production, sales or regulatory compliance resources. There is intense competition among major pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions for qualified personnel in the areas of our activities, however, and we may be unsuccessful in attracting and retaining these personnel.

**Trends toward managed health care and downward price pressures on medical products and services may limit our ability to profitably sell any drugs that we may develop.**

Lower prices for pharmaceutical products may result from:

- third-party-payers' increasing challenges to the prices charged for medical products and services;
- the trend toward managed health care in the U.S. and the concurrent growth of HMOs and similar organizations that can control or significantly influence the purchase of healthcare services and products; and
- legislative proposals to reform healthcare or reduce government insurance programs.

The cost containment measures that healthcare providers are instituting, including practice protocols and guidelines and clinical pathways, and the effect of any healthcare reform, could limit our ability to profitably sell any drugs that we may successfully develop. Moreover, any future legislation or regulation, if any, relating to the healthcare industry or third-party coverage and reimbursement, may cause our business to suffer.

**Security breaches and other disruptions could compromise our information and expose us to liability, which would cause our business and reputation to suffer.**

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers and business partners, as well as personally identifiable information of clinical trial participants and employees. Similarly, our business partners and third party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information, including our data being breached at our business partners or third-party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation which could adversely affect our business.

***Risks Related to our Intellectual Property*****It is difficult and costly to protect our proprietary rights, and we may not be able to ensure protection of such rights.**

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our product candidates, and the methods used to manufacture them, as well as successfully defending these patents against third-party challenges. We will only be able to protect our product candidates from unauthorized making, using, selling and offering to sell or importation by third parties to the extent that we have rights under valid and enforceable patents or trade secrets that cover these activities. The patent positions of pharmaceutical and biotechnology companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the U.S. The biotechnology patent situation outside the U.S. is even more uncertain. Changes in either the patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our issued patents or in third-party patents.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to produce compounds or molecules that are competitive with our product candidates but that are not covered by the claims of our patents;
- we may not have been the first to make the inventions covered by our pending patent applications;
- we may not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents and it is possible that our issued patents could be narrowed in scope, invalidated, held to be unenforceable, or circumvented;
- we may not develop additional proprietary technologies that are patentable; or
- the patents of others may have an adverse effect on our business; or others may be able to misappropriate our trade secrets.

We also may rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. While we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific

collaborators and other advisors may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third party illegally obtained and is using our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

**We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights and we may be unable to protect our rights to, or use, our technology.**

If we choose to go to court to stop someone else from using the inventions claimed in our patents, that individual or company has the right to ask the court to rule that these patents are invalid and/or should not be enforced against that third party. These lawsuits are expensive and would consume time and other resources even if we were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we do not have the right to stop the other party from using the inventions. There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to these patents.

Furthermore, a third party may claim that we are using inventions covered by the third party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and technical personnel. There is a risk that a court would decide that we are infringing the third party's patents and would order us to stop the activities covered by the patents. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. The biotechnology industry has produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our products or methods of use either do not infringe the patent claims of the relevant patent and/or that the patent claims are invalid, and we may not be able to do this. Proving invalidity, in particular, is difficult since it requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Because some patent applications in the U.S. may be maintained in secrecy until the patents are issued, patent applications in the U.S. and many foreign jurisdictions are typically not published until eighteen months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our issued patents or our pending applications or that we were the first to invent the technology. Our competitors have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications and could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the PTO, to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful, resulting in a loss of our United States patent position with respect to such inventions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

**Future litigation, including product liability claims, private securities litigation, stockholder derivative suits and contract litigation, may adversely affect our financial condition and results of operations or liquidity.**

The development, manufacture and marketing of pharmaceutical products of the types that we produce entail an inherent risk of product liability claims. A number of factors could result in an unsafe condition or injury to a patient with respect to these or other products that we manufacture or sell, including inadequate disclosure of product-related risks or product-related information. In addition, we may be the subject of litigation involving contract disputes, stockholder derivative suits or private

securities litigation. The outcome of litigation, particularly class action lawsuits, is difficult to assess or quantify. Plaintiffs in these types of lawsuits often seek recovery of very large or indeterminate amounts, including not only actual damages, but also punitive damages. The magnitude of the potential losses relating to these lawsuits may remain unknown for substantial periods of time. In addition, the cost to defend against any future litigation may be significant. Product liability claims, securities and commercial litigation and other litigation in the future, regardless of the outcome, could have a material adverse effect on our financial condition, results of operations or liquidity.

**We may not be successful in protecting our intellectual property and proprietary rights.**

Our success depends, in part, on our ability to obtain U.S. and foreign patent protection for our drug candidates and processes, preserve our trade secrets and operate our business without infringing the proprietary rights of third parties. Legal standards relating to the validity of patents covering pharmaceutical and biotechnological inventions and the scope of claims made under such patents are still developing and there is no consistent policy regarding the breadth of claims allowed in biotechnology patents. The patent position of a biotechnology firm is highly uncertain and involves complex legal and factual questions. We cannot assure you that any existing or future patents issued to, or licensed by, us will not subsequently be challenged, infringed upon, invalidated or circumvented by others. We cannot assure you that any patents will be issued from any of the patent applications owned by, or licensed to, us. Furthermore, any rights that we may have under issued patents may not provide us with significant protection against competitive products or otherwise be commercially viable.

Patents may have been granted to third parties or may be granted covering products or processes that are necessary or useful to the development of our drug candidates. If our drug candidates or processes are found to infringe upon the patents or otherwise impermissibly utilize the intellectual property of others, our development, manufacture and sale of such drug candidates could be severely restricted or prohibited. In such event, we may be required to obtain licenses from third parties to utilize the patents or proprietary rights of others. We cannot assure you that we will be able to obtain such licenses on acceptable terms, if at all. If we become involved in litigation regarding our intellectual property rights or the intellectual property rights of others, the potential cost of such litigation, regardless of the strength of our legal position, and the potential damages that we could be required to pay could be substantial.

**Our products could infringe on the intellectual property rights of others, and we may be required to license technology from third parties in the future in order to market our products.**

Companies in the biotechnology and pharmaceutical industries steadfastly pursue and protect intellectual property rights. This can result in considerable and costly litigation to determine the validity of patents and claims by third parties of infringement of patents or other intellectual property. Our gene therapy products could be found to infringe on the intellectual property rights of others. Other companies may hold or obtain patents or inventions or other proprietary rights in technology necessary for our business. We have or may be required to obtain licenses from other companies to use such proprietary rights. We may be unable to obtain licenses to use such proprietary rights. Furthermore, should we violate the terms of a license, that license could be cancelled. Our ability to achieve profitability and positive cash flow may be negatively affected by our inability to procure such a license, the cancellation of any such license, any new license fees arising out of any new license, or any increases in license fees we currently pay. Periodically companies inquire about our products and technology in their attempts to assess whether we violate their intellectual property rights. If we are forced to defend against infringement claims, we may face costly litigation and diversion of technical and management personnel, even if the allegations of infringement are unwarranted. In addition, as a result of potential infringement claims, we may be required to obtain one or more licenses from other companies to use the infringed technology, and the license fees we pay may negatively affect our ability to achieve profitability and positive cash flow. If there is a successful claim of infringement against us and we are unable to develop non-infringing technology or license the infringed or similar technology on a timely basis, our business, and our ability to grow revenue and achieve profitability and positive cash flow, could be adversely affected.

We are aware of an issued U.S. patent owned by a third party directed to the AAV9 viral vector and methods for its use. This patent was issued in 2002. We understand that the owner of this patent grants licenses under the patent from time to time, and we expect that, prior to commercializing our ABO-101 or

ABO-102 product candidates (which utilize the AAV9 vector), we would seek to license this patent. There can be no assurance that we will be successful in licensing this patent or that any such patent would be on favorable terms to us. Other licensed technologies may also require the licensing of additional patents for commercialization.

**We are substantially dependent on technologies we license from Nationwide Children’s Hospital for ABO-101 and ABO-102, and if we lose the license to such technologies or the applicable license is terminated for any reason, our ability to develop ABO-101 and ABO-102 would be harmed, and our business, financial condition and results of operations would be materially and adversely affected.**

Our business is substantially dependent upon technology licensed from Nationwide Children’s Hospital. Pursuant to the Nationwide Children’s Hospital License Agreement, we have been granted exclusive rights for the intellectual property for ABO-101 and ABO-102. All of the intellectual property related to our ABO-101 and ABO-102 is currently owned by Nationwide Children’s Hospital, and we have the rights to use such intellectual property pursuant to the Nationwide Children’s Hospital License Agreement. Therefore, our ability to develop and commercialize our drug candidates depends entirely on the effectiveness and continuation of the Nationwide Children’s Hospital agreement. If we lose the right to license any of these key compounds, our ability to develop existing and new drug candidates would be harmed.

Nationwide Children’s Hospital has the right to terminate the Nationwide Children’s Hospital License Agreement under certain circumstances, including, but not limited to: (1) in the event of our insolvency or bankruptcy, (2) written notice with at least six months’ notice, or (3) if we default on certain of our material obligations and fail to cure the default within a specified period of time.

#### ***Risks Related to our Common Stock***

**The market price of our common stock may be volatile and adversely affected by several factors.**

The market price of our common stock could fluctuate significantly in response to various factors and events, including:

- our ability to integrate operations, technology, products and services;
- our ability to execute our business plan;
- operating results below expectations;
- announcements concerning product development results, including clinical trial results, or intellectual property rights of others;
- litigation or public concern about the safety of our potential products;
- our issuance of additional securities, including debt or equity or a combination thereof, which will be necessary to fund our operating expenses;
- announcements of technological innovations or new products by us or our competitors;
- loss of any strategic relationship;
- industry developments, including, without limitation, changes in healthcare policies or practices or third-party reimbursement policies; economic and other external factors;
- period-to-period fluctuations in our financial results; and
- whether an active trading market in our common stock develops and is maintained.

In addition, the securities markets have from time to time experienced significant price and volume fluctuations that are unrelated to the operating performance of particular companies. These market fluctuations may also materially and adversely affect the market price of our common stock.

**We have not paid cash dividends in the past and do not expect to pay cash dividends in the foreseeable future. Any return on investment may be limited to the value of our common stock.**

We have never paid cash dividends on our common stock and do not anticipate paying cash dividends on our common stock in the foreseeable future. The payment of dividends on our capital stock will depend on our earnings, financial condition and other business and economic factors affecting us at such time as the board of directors may consider relevant. If we do not pay dividends, our common stock may be less valuable because a return on your investment will only occur if the common stock price appreciates.

**Our quarterly operating results may fluctuate significantly.**

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our development programs;
- addition or termination of clinical trials;
- any intellectual property infringement lawsuit in which we may become involved;
- regulatory developments affecting our product candidates; and
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our common stock to fluctuate substantially.

**Provisions of our charter documents could delay or discourage an acquisition of our Company that would benefit our stockholders and may have the effect of making it difficult to remove management.**

Provisions of our Certificate of Incorporation and By-laws may make it more difficult for a third party to acquire control of us, even if a change in control would benefit our stockholders. In particular, shares of our preferred stock may be issued in the future without further stockholder approval and upon such terms and conditions, and having such rights, privileges and preferences, as our Board of Directors may determine, including, for example, rights to convert into our common stock. The rights of the holders of our common stock will be subject to, and may be adversely affected by, the rights of the holders of any of our preferred stock that may be issued in the future. The issuance of our preferred stock, while providing desirable flexibility in connection with possible acquisitions and other corporate purposes, could have the effect of making it more difficult for a third party to acquire control of us. This could limit the price that certain investors might be willing to pay in the future for shares of our common stock and discourage these investors from acquiring a majority of our common stock. Further, the existence of these corporate governance provisions could have the effect of making it more difficult to change our management.

**Failure to achieve and maintain effective internal controls could have a material adverse effect on our business.**

Effective internal controls are necessary for us to provide reliable financial reports. If we cannot provide reliable financial reports, our operating results could be harmed. All internal control systems, no matter how well designed, have inherent limitations. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation.

Based on our evaluation, our management concluded that there is no material weakness in our internal control over financial reporting for the year ended December 31, 2017 based on the criteria established in Internal Control-Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (“COSO”).

While we continue to evaluate and improve our internal controls, we cannot be certain that these measures will ensure adequate controls over our financial processes and reporting in the future. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could harm our operating results or cause us to fail to meet our reporting obligations. Failure to achieve and maintain an effective internal control environment could cause investors to lose confidence in our reported financial information, which could have a material adverse effect on our stock price. Failure to comply with Section 404 of the Sarbanes-Oxley Act of 2002 could also potentially subject us to sanctions or investigations by the SEC or other regulatory authorities.

**There can be no assurance that we will be able to comply with continued listing standards of the Nasdaq Capital Market.**

We cannot assure you that we will be able to continue to comply with the minimum bid price and the other standards that we are required to meet in order to maintain a listing of our common stock on the Nasdaq Capital Market. Our failure to continue to meet these requirements may result in our common stock being delisted from the Nasdaq Capital Market.

**Our ability to use our net operating loss carry forwards may be subject to limitation.**

Generally, a change of more than 50% in the ownership of a company's stock, by value, over a three-year period constitutes an ownership change for U.S. federal income tax purposes. An ownership change may limit our ability to use our net operating loss carryforwards attributable to the period prior to the change. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may become subject to limitations, which could potentially result in increased future tax liability for us. At December 31, 2017, we had net operating loss carryforwards aggregating approximately \$238.2 million.

**Ownership of our shares is concentrated in the hands of a few investors which could limit the ability of our other stockholders to influence the direction of the Company.**

As calculated by SEC rules of beneficial ownership, SCO Capital Partners LLC and affiliates beneficially owned approximately 28.4% of our common stock as of May 10, 2018. Accordingly, they collectively have the ability to significantly influence or determine the election of all of our directors and the outcome of most corporate actions requiring stockholder approval. They may exercise this ability in a manner that advances their best interests and not necessarily those of our other stockholders.

### **CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This prospectus, any prospectus supplement and the other documents we have filed with the SEC that are incorporated herein by reference contain forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans, objectives of management or other financial items are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “seek,” “intend,” “may,” “plan,” “predict,” “project,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly as set forth and incorporated by reference into the “Risk Factors” section above, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

You should read this prospectus, any supplements to this prospectus and the documents that we incorporate by reference into this prospectus completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, except as otherwise required by law. We advise you, however, to consult any further disclosures we make on related subjects in our future annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K we file with or furnish to the SEC.

### **USE OF PROCEEDS**

Unless otherwise specified in a prospectus supplement accompanying this prospectus, the net proceeds from the sale of the securities to which this prospectus relates will be used for general corporate purposes. General corporate purposes may include repayment of debt, acquisitions, additions to working capital, capital expenditures, research and development, and investments in our subsidiaries. Net proceeds may be temporarily invested prior to use.

## PLAN OF DISTRIBUTION

We may sell the offered securities in any of the ways described below or in any combination or any other way set forth in an applicable prospectus supplement from time to time:

- to or through underwriters or dealers;
- through one or more agents; or
- directly to purchasers or to a single purchaser.

The distribution of the securities may be effected from time to time in one or more transactions:

- at a fixed price, or prices, which may be changed from time to time;
- at market prices prevailing at the time of sale;
- at prices related to such prevailing market prices; or
- at negotiated prices.

Each prospectus supplement will describe the method of distribution of the securities and any applicable restrictions.

The prospectus supplement with respect to the securities of a particular series will describe the terms of the offering of the securities, including the following:

- the name or names of any underwriters, dealers or agents and the amounts of securities underwritten or purchased by each of them;
- the public offering price of the securities and the proceeds to us and any discounts, commissions or concessions allowed or reallocated or paid to dealers; and
- any securities exchanges on which the securities may be listed.

Any offering price and any discounts or concessions allowed or reallocated or paid to dealers may be changed from time to time.

Only the agents or underwriters named in each prospectus supplement are agents or underwriters in connection with the securities being offered thereby.

We may authorize underwriters, dealers or other persons acting as our agents to solicit offers by certain institutions to purchase securities from us pursuant to delayed delivery contracts providing for payment and delivery on the date stated in each applicable prospectus supplement. Each contract will be for an amount not less than, and the aggregate amount of securities sold pursuant to such contracts shall not be less nor more than, the respective amounts stated in each applicable prospectus supplement. Institutions with whom the contracts, when authorized, may be made include commercial and savings banks, insurance companies, pension funds, investment companies, educational and charitable institutions and other institutions, but shall in all cases be subject to our approval. Delayed delivery contracts will be subject only to those conditions set forth in each applicable prospectus supplement, and each prospectus supplement will set forth any commissions we pay for solicitation of these contracts.

Agents, underwriters and other third parties described above may be entitled to indemnification by us against certain civil liabilities, including liabilities under the Securities Act of 1933, as amended (the “Securities Act”), or to contribution from us with respect to payments which the agents, underwriters or other third parties may be required to make in respect thereof. Agents, underwriters and such other third parties may be customers of, engage in transactions with, or perform services for us in the ordinary course of business.

One or more firms, referred to as “remarketing firms,” may also offer or sell the securities, if a prospectus supplement so indicates, in connection with a remarketing arrangement upon their purchase. Remarketing firms will act as principals for their own accounts or as our agents. These remarketing firms will offer or sell the securities in accordance with the terms of the securities. Each prospectus supplement will identify and describe any remarketing firm and the terms of its agreement, if any, with us and will describe the remarketing firm’s compensation. Remarketing firms may be deemed to be underwriters in

connection with the securities they remarket. Remarketing firms may be entitled under agreements that may be entered into with us to indemnification by us against certain civil liabilities, including liabilities under the Securities Act, and may be customers of, engage in transactions with or perform services for us in the ordinary course of business.

Certain underwriters may use this prospectus and any accompanying prospectus supplement for offers and sales related to market-making transactions in the securities. These underwriters may act as principal or agent in these transactions, and the sales will be made at prices related to prevailing market prices at the time of sale.

The securities we offer may be new issues of securities and may have no established trading market. The securities may or may not be listed on a securities exchange. Underwriters may make a market in these securities, but will not be obligated to do so and may discontinue any market making at any time without notice. We can make no assurance as to the liquidity of, or the existence of trading markets for, any of the securities.

Certain persons participating in an offering may engage in over-allotment, stabilizing transactions, short covering transactions and penalty bids in accordance with rules and regulations under the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Over-allotment involves sales in excess of the offering size, which create a short position. Stabilizing transactions permit bids to purchase the underlying security so long as the stabilizing bids do not exceed a specified maximum. Short covering transactions involve purchases of the securities in the open market after the distribution is completed to cover short positions. Penalty bids permit the underwriters to reclaim a selling concession from a dealer when the securities originally sold by the dealer are purchased in a short covering transaction to cover short positions. Those activities may cause the price of the securities to be higher than it would otherwise be. If commenced, the underwriters may discontinue any of the activities at any time.

We also may sell any of the securities through agents designated by us from time to time. We will name any agent involved in the offer or sale of these securities and will list commissions payable by us to these agents in the applicable prospectus supplement. These agents will be acting on a best efforts basis to solicit purchases for the period of its appointment, unless stated otherwise in the applicable prospectuses.

We may sell any of the securities directly to purchasers. In this case, we will not engage underwriters or agents in the offer and sale of these securities.

We may engage in sales deemed to be "at the market offerings" as defined in Rule 415 promulgated under the Securities Act, including sales made directly on or through the Nasdaq Capital Market, the existing trading market for our common stock, sales made to or through a market maker other than on an exchange or otherwise, in negotiated transactions at market prices prevailing at the time of sale or at prices related to such prevailing market prices, and/or any other method permitted by law. The terms of such "at the market offerings" will be set forth in the applicable prospectus supplement. We may engage an agent to act as a sales agent in such "at the market offerings" on a best efforts basis using commercially reasonable efforts consistent with normal trading and sales practices, on mutually agreed terms between such agent and us. We will name any agent involved in such "at the market offerings" of securities and will list commissions payable by us to these agents in the applicable prospectus supplement.

In addition, we may enter into derivative transactions with third parties, or sell securities not covered by this prospectus to third parties in privately negotiated transactions. If the applicable prospectus supplement so indicates, in connection with those derivatives, the third parties may sell securities covered by this prospectus and the applicable prospectus supplement, including in short sale transactions. If so, the third party may use securities pledged by us or borrowed from us or others to settle those sales or to close out any related open borrowings of stock, and may use securities received from us in settlement of those derivatives to close out any related open borrowings of stock. The third party in such sale transactions will be an underwriter and, if not identified in this prospectus, will be named in the applicable prospectus supplement (or a post-effective amendment). In addition, we may otherwise loan or pledge securities to a financial institution or other third party that in turn may sell the securities short using this prospectus and an applicable prospectus supplement. Such financial institution or other third party may transfer its economic short position to investors in our securities or in connection with a concurrent offering of other securities.

The specific terms of any lock-up provisions in respect of any given offering will be described in the applicable prospectus supplement.

In compliance with the guidelines of the Financial Industry Regulatory Authority, Inc., or FINRA, the maximum consideration or discount to be received by any FINRA member or independent broker dealer may not exceed 8% of the aggregate proceeds of the offering.

The underwriters, dealers and agents may engage in transactions with us, or perform services for us, in the ordinary course of business for which they receive compensation.

**GENERAL DESCRIPTION OF SECURITIES THAT WE MAY SELL**

We may offer and sell, at any time and from time to time:

- Shares of our common stock;
- Shares of our preferred stock;
- Warrants to purchase shares of our common stock, preferred stock and/or debt securities;
- Debt securities consisting of debentures, notes or other evidences of indebtedness; or
- Any combination of these securities.

The terms of any securities we offer will be determined at the time of sale. We may issue debt securities that are exchangeable for or convertible into common stock or any of the other securities that may be sold under this prospectus. When particular securities are offered, a supplement to this prospectus will be filed with the SEC, which will describe the terms of the offering and sale of the offered securities.

## DESCRIPTION OF OUR COMMON STOCK

Our authorized capital stock consists of 200,000,000 shares of common stock, \$0.01 par value per share, and 2,000,000 shares of preferred stock, \$0.01 par value per share, which may be issued in one or more series. The following summary of the terms of our common stock is subject to and qualified in its entirety by reference to our charter and by-laws, copies of which are on file with the SEC as exhibits to previous SEC filings. Please refer to “Where You Can Find More Information” below for directions on obtaining these documents.

As of May 11, 2018, we had 47,305,285 shares of common stock outstanding and no shares of preferred stock outstanding.

### General

Holder of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and have the right to vote cumulatively for the election of directors. This means that in the voting at our annual meeting, each stockholder or his proxy, may multiply the number of his shares by the number of directors to be elected then cast the resulting total number of votes for a single nominee, or distribute such votes on the ballot among the nominees as desired. Holders of our common stock are entitled to receive ratably such dividends, if any, as may be declared by our Board of Directors out of funds legally available therefor, subject to any preferential dividend rights for our outstanding preferred stock.

Upon our liquidation, dissolution or winding up, the holders of our common stock are entitled to receive ratably our net assets available after the payment of all debts and other liabilities and subject to the prior rights of any of our outstanding preferred stock. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock which we may designate and issue in the future.

Our common stockholders may not receive any assets or funds until our creditors have been paid in full and the preferential or participating rights of our preferred stockholders have been satisfied. If we participate in a corporate merger, consolidation, purchase or acquisition of property or stock, or other reorganization, any payments or shares of stock allocated to our common stockholders will be distributed pro rata to holders of our common stock on a per share basis. If we redeem, repurchase or otherwise acquire for payment any shares of our common stock, we will treat each share of common stock identically.

We may issue additional shares of our common stock, if authorized by the Board, without the common stockholders’ approval, unless required by Delaware law or a stock exchange on which our securities are traded. If we receive the appropriate payment, shares of our common stock that we issue will be fully paid and nonassessable.

### Anti-Takeover Provisions

We are subject to the provisions of Section 203 of the General Corporation Law of Delaware. Section 203 prohibits certain publicly held Delaware corporations from engaging in a “business combination” with an “interested stockholder,” for a period of three years after the date of the transaction in which the person became an “interested stockholder,” unless the business combination is approved in a prescribed manner. A “business combination” includes mergers, asset sales and other transactions resulting in a financial benefit to the interested stockholder. Subject to certain exceptions, an “interested stockholder” is a person or entity who, together with affiliates and associates, owns (or within the preceding three years, did own) 15% or more of the corporation’s voting stock. The statute contains provisions enabling a corporation to avoid the statute’s restrictions if the stockholders holding a majority of the corporation’s voting stock approve the transaction. Moreover, our certificate of incorporation provides that our directors shall be divided into three classes, with the terms of each class to expire on different years.

In addition, our certificate of incorporation, in order to combat “greenmail,” provides in general that any direct or indirect purchase by us of any of our voting stock or rights to acquire voting stock known to be beneficially owned by any person or group which holds more than five percent of a class of our voting stock and which has owned the securities being purchased for less than two years must be approved by the

affirmative vote of at least two-thirds of the votes entitled to be cast by the holders of voting stock, subject to certain exceptions. The prohibition of “greenmail” may tend to discourage or foreclose certain acquisitions of our securities which might temporarily increase the price of our securities. Discouraging the acquisition of a large block of our securities by an outside party may also have a potential negative effect on takeovers. Parties seeking control of us through large acquisitions of its securities will not be able to resort to “greenmail” should their bid fail, thus making such a bid less attractive to persons seeking to initiate a takeover effort.

**Elimination of Monetary Liability for Officers and Directors**

Our certificate of incorporation incorporates certain provisions permitted under the General Corporation Law of Delaware relating to the liability of directors. The provisions eliminate a director’s liability for monetary damages for a breach of fiduciary duty, including gross negligence, except in circumstances involving certain wrongful acts, such as the breach of director’s duty of loyalty or acts or omissions, which involve intentional misconduct or a knowing violation of law. These provisions do not eliminate a director’s duty of care. Moreover, these provisions do not apply to claims against a director for certain violations of law, including knowing violations of federal securities law. Our Certificate of Incorporation also contains provisions to indemnify the directors, officers, employees or other agents to the fullest extent permitted by the General Corporation Law of Delaware. We believe that these provisions will assist us in attracting and retaining qualified individual to serve as directors.

Our Certificate of Incorporation also contains provisions to indemnify the directors, officers, employees or other agents to the fullest extent permitted by the General Corporation Law of Delaware. These provisions may have the practical effect in certain cases of eliminating the ability of stockholders to collect monetary damages from directors. We believe that these provisions will assist us in attracting or retaining qualified individuals to serve as our directors.

**The Nasdaq Capital Market**

Our common stock is listed on the Nasdaq Capital Market under the symbol “ABEO.”

**Transfer Agent and Registrar**

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, New York, New York.

### DESCRIPTION OF OUR PREFERRED STOCK

The Board may, without further action by our stockholders, from time to time, direct the issuance of shares of preferred stock in series and may, at the time of issuance, determine the rights, preferences and limitations of each series, including voting rights, dividend rights and redemption and liquidation preferences. Satisfaction of any dividend preferences of outstanding shares of our preferred stock would reduce the amount of funds available for the payment of dividends on shares of our common stock. Holders of shares of our preferred stock may be entitled to receive a preference payment in the event of any liquidation, dissolution or winding-up of our Company before any payment is made to the holders of shares of our common stock. In some circumstances, the issuance of shares of preferred stock may render more difficult or tend to discourage a merger, tender offer or proxy contest, the assumption of control by a holder of a large block of our securities or the removal of incumbent management. Upon the affirmative vote of the Board, without stockholder approval, we may issue shares of preferred stock with voting and conversion rights which could adversely affect the holders of shares of our common stock.

If we offer a specific class or series of preferred stock under this prospectus, we will describe the terms of the preferred stock in the prospectus supplement for such offering and will file a copy of the certificate establishing the terms of the preferred stock with the SEC. To the extent required, this description will include:

- the title and stated value;
- the number of shares offered, the liquidation preference per share and the purchase price;
- the dividend rate(s), period(s) and/or payment date(s), or method(s) of calculation for such dividends;
- whether dividends will be cumulative or non-cumulative and, if cumulative, the date from which dividends will accumulate;
- the procedures for any auction and remarketing, if any;
- the provisions for a sinking fund, if any;
- the provisions for redemption, if applicable;
- any listing of the preferred stock on any securities exchange or market;
- whether the preferred stock will be convertible into our common stock, and, if applicable, the conversion price (or how it will be calculated), the conversion period and any other terms of conversion (including any anti-dilution provisions, if any);
- whether the preferred stock will be exchangeable into debt securities, and, if applicable, the exchange price (or how it will be calculated), the exchange period and any other terms of exchange (including any anti-dilution provisions, if any);
- voting rights, if any, of the preferred stock;
- a discussion of any material U.S. federal income tax considerations applicable to the preferred stock;
- the relative ranking and preferences of the preferred stock as to dividend rights and rights upon liquidation, dissolution or winding up of the affairs of the Company;
- any material limitations on issuance of any class or series of preferred stock ranking senior to or on a parity with the series of preferred stock as to dividend rights and rights upon liquidation, dissolution or winding up of the Company; and
- any other affirmative, negative or other covenants or contractual rights which might be attendant with the specific class or series of preferred stock.

The preferred stock offered by this prospectus, when issued, will not have, or be subject to, any preemptive or similar rights.

### **Transfer Agent and Registrar**

The transfer agent and registrar for any series or class of preferred stock will be set forth in each applicable prospectus supplement.

### **DESCRIPTION OF OUR WARRANTS**

This section describes the general terms and provisions of our warrants to acquire our securities that we may issue from time to time. The applicable prospectus supplement will describe the specific terms of the warrants offered through that prospectus supplement.

We may issue warrants for the purchase of our debt securities, common stock or preferred stock or other securities issued by us. We may issue warrants independently or together with other securities, and they may be attached to or separate from the other securities. We will file a copy of the warrant and warrant agreement with the SEC each time we issue a series of warrants, and these warrants and warrant agreements will be incorporated by reference into the Registration Statement of which this prospectus is a part. A holder of our warrants should refer to the provisions of the applicable warrant agreement and prospectus supplement for more specific information.

The applicable prospectus supplement will contain, where applicable, the following terms of and other information relating to the warrants:

- the specific designation and aggregate number of, and the price at which we will issue, the warrants;
- the currency or currency units in which the offering price, if any, and the exercise price are payable;
- the designation, amount and terms of the securities purchasable upon exercise of the warrants;
- if applicable, the exercise price for shares of our common stock and the number of shares of common stock to be received upon exercise of the warrants;
- if applicable, the exercise price for shares of our preferred stock, the number of shares of preferred stock to be received upon exercise, and a description of that class or series of our preferred stock;
- if applicable, the exercise price for our debt securities, the amount of our debt securities to be received upon exercise, and a description of that series of debt securities;
- the date on which the right to exercise the warrants will begin and the date on which that right will expire or, if the warrants may not be continuously exercised throughout that period, the specific date or dates on which the warrants may be exercised;
- whether the warrants will be issued in fully registered form or bearer form, in definitive or global form or in any combination of these forms, although, in any case, the form of a warrant included in a unit will correspond to the form of the unit and of any security included in that unit;
- any applicable material U.S. federal income tax consequences;
- the identity of the warrant agent for the warrants and of any other depositaries, execution or paying agents, transfer agents, registrars or other agents;
- the proposed listing, if any, of the warrants or any securities purchasable upon exercise of the warrants on any securities exchange;
- if applicable, the date from and after which the warrants and the common stock, preferred stock or debt securities will be separately transferable;
- if applicable, the minimum or maximum amount of the warrants that may be exercised at any one time;
- information with respect to book-entry procedures, if any;
- the anti-dilution provisions of the warrants, if any;

- any redemption or call provisions;
- whether the warrants are to be sold separately or with other securities as parts of units; and
- any additional terms of the warrants, including terms, procedures and limitations relating to the exchange and exercise of the warrants.

After your warrants expire they will become void. All warrants will be issued in registered form. The prospectus supplement may provide for the adjustment of the exercise price of the warrants.

Warrants may be exercised at the appropriate office of the warrant agent or any other office indicated in the applicable prospectus supplement. Before the exercise of warrants, holders will not have any of the rights of holders of the securities purchasable upon exercise and will not be entitled to payments made to holders of those securities.

The warrant agreements may be amended or supplemented without the consent of the holders of the warrants to which it applies to effect changes that are not inconsistent with the provisions of the warrants and that do not materially and adversely affect the interests of the holders of the warrants. However, any amendment that materially and adversely alters the rights of the holders of warrants will not be effective unless the holders of at least a majority of the applicable warrants then outstanding approve the amendment. Every holder of an outstanding warrant at the time any amendment becomes effective, by continuing to hold the warrant, will be bound by the applicable warrant agreement as amended. The prospectus supplement applicable to a particular series of warrants may provide that certain provisions of the warrants, including the securities for which they may be exercisable, the exercise price and the expiration date, may not be altered without the consent of the holder of each warrant.

#### **Transfer Agent and Registrar**

The transfer agent and registrar for any warrants will be set forth in the applicable prospectus supplement.

### **DESCRIPTION OF OUR DEBT SECURITIES**

This section describes the general terms and provisions of the debt securities that we may offer under this prospectus, any of which may be issued as convertible or exchangeable debt securities. We will set forth the particular terms of the debt securities we offer in a prospectus supplement. The extent, if any, to which the following general provisions apply to particular debt securities will be described in the applicable prospectus supplement. The following description of general terms relating to the debt securities and the indenture under which the debt securities will be issued are summaries only and therefore are not complete. You should read the indenture and the prospectus supplement regarding any particular issuance of debt securities.

We will issue any debt under an indenture to be entered into between us and the trustee identified in the applicable prospectus supplement. The terms of the debt securities will include those stated in the indenture and those made part of the indenture by reference to the Trust Indenture Act of 1939, as amended (the “Indenture Act”), as in effect on the date of the indenture. We have filed or will file a copy of the form of indenture as an exhibit to the Registration Statement in which this prospectus is included. The indenture will be subject to and governed by the terms of the Indenture Act.

We may offer under this prospectus up to an aggregate principal amount of \$500,000,000 in debt securities, or if debt securities are issued at a discount, or in a foreign currency, foreign currency units or composite currency, the principal amount as may be sold for an initial public offering price of up to \$500,000,000. Unless otherwise specified in the applicable prospectus supplement, the debt securities will represent direct, unsecured obligations of the Company and will rank equally with all of our other unsecured indebtedness.

The following statements relating to the debt securities and the indenture are summaries, qualified in their entirety by reference to the detailed provisions of the indenture and the final form indenture as may be filed with a future prospectus supplement.

## General

We may issue the debt securities in one or more series with the same or various maturities, at par, at a premium, or at a discount. We will describe the particular terms of each series of debt securities in a prospectus supplement relating to that series, which we will file with the SEC.

The prospectus supplement will set forth, to the extent required, the following terms of the debt securities in respect of which the prospectus supplement is delivered:

- the title of the series;
- the aggregate principal amount;
- the issue price or prices, expressed as a percentage of the aggregate principal amount of the debt securities;
- any limit on the aggregate principal amount;
- the date or dates on which principal is payable;
- the interest rate or rates (which may be fixed or variable) or, if applicable, the method used to determine such rate or rates;
- the date or dates from which interest, if any, will be payable and any regular record date for the interest payable;
- the place or places where principal and, if applicable, premium and interest, is payable;
- the terms and conditions upon which we may, or the holders may require us to, redeem or repurchase the debt securities;
- the denominations in which such debt securities may be issuable, if other than denominations of \$1,000 or any integral multiple of that number;
- whether the debt securities are to be issuable in the form of certificated securities (as described below) or global securities (as described below);
- the portion of principal amount that will be payable upon declaration of acceleration of the maturity date if other than the principal amount of the debt securities;
- the currency of denomination;
- the designation of the currency, currencies or currency units in which payment of principal and, if applicable, premium and interest, will be made;
- if payments of principal and, if applicable, premium or interest, on the debt securities are to be made in one or more currencies or currency units other than the currency of denomination, the manner in which the exchange rate with respect to such payments will be determined;
- if amounts of principal and, if applicable, premium and interest may be determined by reference to an index based on a currency or currencies or by reference to a commodity, commodity index, stock exchange index or financial index, then the manner in which such amounts will be determined;
- the provisions, if any, relating to any collateral provided for such debt securities;
- any addition to or change in the covenants and/or the acceleration provisions described in this prospectus or in the indenture;
- any events of default, if not otherwise described below under “Default and Notice”;
- the terms and conditions, if any, for conversion into or exchange for shares of our common stock or preferred stock;

- any depositaries, interest rate calculation agents, exchange rate calculation agents or other agents; and
- the terms and conditions, if any, upon which the debt securities shall be subordinated in right of payment to other indebtedness of the Company.

We may issue discount debt securities that provide for an amount less than the stated principal amount to be due and payable upon acceleration of the maturity of such debt securities in accordance with the terms of the indenture. We may also issue debt securities in bearer form, with or without coupons. If we issue discount debt securities or debt securities in bearer form, we will describe material U.S. federal income tax considerations and other material special considerations which apply to these debt securities in the applicable prospectus supplement.

We may issue debt securities denominated in or payable in a foreign currency or currencies or a foreign currency unit or units. If we do, we will describe the restrictions, elections, and general tax considerations relating to the debt securities and the foreign currency or currencies or foreign currency unit or units in the applicable prospectus supplement.

#### **Exchange and/or Conversion Rights**

We may issue debt securities which can be exchanged for or converted into shares of our common stock or preferred stock. If we do, we will describe the terms of exchange or conversion in the prospectus supplement relating to these debt securities.

#### **Transfer and Exchange**

We may issue debt securities that will be represented by either:

- “book-entry securities,” which means that there will be one or more global securities registered in the name of a depositary or a nominee of a depositary; or
- “certificated securities,” which means that they will be represented by a certificate issued in definitive registered form.

We will specify in the prospectus supplement applicable to a particular offering whether the debt securities offered will be book-entry or certificated securities.

#### **Certificated Debt Securities**

If you hold certificated debt securities issued under an indenture, you may transfer or exchange such debt securities in accordance with the terms of the indenture. You will not be charged a service charge for any transfer or exchange of certificated debt securities but may be required to pay an amount sufficient to cover any tax or other governmental charge payable in connection with such transfer or exchange.

#### **Global Securities**

The debt securities of a series may be issued in the form of one or more global securities that will be deposited with a depositary or its nominees identified in the prospectus supplement relating to the debt securities. In such a case, one or more global securities will be issued in a denomination or aggregate denominations equal to the portion of the aggregate principal amount of outstanding debt securities of the series to be represented by such global security or securities.

Unless and until it is exchanged in whole or in part for debt securities in definitive registered form, a global security may not be registered for transfer or exchange except as a whole by the depositary for such global security to a nominee of the depositary and except in the circumstances described in the prospectus supplement relating to the debt securities. The specific terms of the depositary arrangement with respect to a series of debt securities will be described in the prospectus supplement relating to such series.

#### **No Protection in the Event of Change of Control**

Any indenture that governs our debt securities covered by this prospectus may not have any covenant or other provision providing for a put or increased interest or otherwise that would afford holders of our

debt securities additional protection in the event of a recapitalization transaction, a change of control of the Company, or a highly leveraged transaction. If we offer any covenants or provisions of this type with respect to any debt securities covered by this prospectus, we will describe them in the applicable prospectus supplement.

### **Covenants**

Unless otherwise indicated in this prospectus or the applicable prospectus supplement, our debt securities may not have the benefit of any covenant that limits or restricts our business or operations, the pledging of our assets or the incurrence by us of indebtedness. We will describe in the applicable prospectus supplement any material covenants in respect of a series of debt securities.

### **Consolidation, Merger and Sale of Assets**

We may agree in any indenture that governs the debt securities of any series covered by this prospectus that we will not consolidate with or merge into any other person or convey, transfer, sell or lease our properties and assets substantially as an entirety to any person, unless such person and such proposed transaction meets various criteria, which we will describe in detail in the applicable prospectus supplement.

### **Defaults and Notice**

The debt securities of any series will contain events of default to be specified in the applicable prospectus supplement, which may include, without limitation:

- failure to pay the principal of, or premium or make-whole amount, if any, on any debt security of such series when due and payable (whether at maturity, by call for redemption, through any mandatory sinking fund, by redemption at the option of the holder, by declaration or acceleration or otherwise);
- failure to make a payment of any interest on any debt security of such series when due;
- our failure to perform or observe any other covenants or agreements in the indenture with respect to the debt securities of such series;
- certain events relating to our bankruptcy, insolvency or reorganization; and
- certain cross defaults, if and as applicable.

If an event of default with respect to debt securities of any series shall occur and be continuing, we may agree that the trustee or the holders of at least 25% in aggregate principal amount of the then outstanding debt securities of such series may declare the principal amount (or, if the debt securities of such series are issued at an original issue discount, such portion of the principal amount as may be specified in the terms of the debt securities of such series) of all debt securities of such series or such other amount or amounts as the debt securities or supplemental indenture with respect to such series may provide, to be due and payable immediately. Any provisions pertaining to events of default and any remedies associated therewith will be described in the applicable prospectus supplement.

Any indenture that governs our debt securities covered by this prospectus may require that the trustee under such indenture shall, within 90 days after the occurrence of a default, give to holders of debt securities of any series notice of all uncured defaults with respect to such series known to it. However, in the case of a default that results from the failure to make any payment of the principal of, premium or make-whole amount, if any, or interest on the debt securities of any series, or in the payment of any mandatory sinking fund installment with respect to debt securities of such series, if any, the trustee may withhold such notice if it in good faith determines that the withholding of such notice is in the interest of the holders of debt securities of such series. Any terms and provisions relating to the foregoing types of provisions will be described in further detail in the applicable prospectus supplement.

Any indenture that governs our debt securities covered by this prospectus will contain a provision entitling the trustee to be indemnified by holders of debt securities before proceeding to exercise any trust or power under the indenture at the request of such holders. Any such indenture may provide that the holders of at least a majority in aggregate principal amount of the then outstanding debt securities of any

series may direct the time, method and place of conducting any proceedings for any remedy available to the trustee, or of exercising any trust or power conferred upon the trustee with respect to the debt securities of such series. However, the trustee under any such indenture may decline to follow any such direction if, among other reasons, the trustee determines in good faith that the actions or proceedings as directed may not lawfully be taken, would involve the trustee in personal liability or would be unduly prejudicial to the holders of the debt securities of such series not joining in such direction.

Any indenture that governs our debt securities covered by this prospectus may endow the holders of such debt securities to institute a proceeding with respect to such indenture, subject to certain conditions, which will be specified in the applicable prospectus supplement and which may include, that the holders of at least a majority in aggregate principal amount of the debt securities of such series then outstanding make a written request upon the trustee to exercise its power under the indenture, indemnify the trustee and afford the trustee reasonable opportunity to act. Even so, such holders may have an absolute right to receipt of the principal of, premium or make-whole amount, if any, and interest when due, to require conversion or exchange of debt securities if such indenture provides for convertibility or exchangeability at the option of the holder and to institute suit for the enforcement of such rights. Any terms and provisions relating to the foregoing types of provisions will be described in further detail in the applicable prospectus supplement.

#### **Modification of the Indenture**

We and the trustee may modify any indenture that governs our debt securities of any series covered by this prospectus with or without the consent of the holders of such debt securities, under certain circumstances to be described in a prospectus supplement.

#### **Defeasance; Satisfaction and Discharge**

The prospectus supplement will outline the conditions under which we may elect to have certain of our obligations under the indenture discharged and under which the indenture obligations will be deemed to be satisfied.

#### **Regarding the Trustee**

We will identify the trustee and any relationship that we may have with such trustee, with respect to any series of debt securities, in the prospectus supplement relating to the applicable debt securities. You should note that if the trustee becomes a creditor of the Company, the indenture and the Indenture Act limit the rights of the trustee to obtain payment of claims in certain cases, or to realize on certain property received in respect of any such claim, as security or otherwise. The trustee and its affiliates may engage in, and will be permitted to continue to engage in, other transactions with us and our affiliates. If, however, the trustee acquires any “conflicting interest” within the meaning of the Indenture Act, it must eliminate such conflict or resign.

#### **Governing Law**

The law governing the indenture and the debt securities will be identified in the prospectus supplement relating to the applicable indenture and debt securities.

## WHERE YOU CAN FIND MORE INFORMATION; INCORPORATION BY REFERENCE

We file annual, quarterly and current reports, proxy statements and other information with the SEC. You may read and copy information filed by us with the SEC at the SEC's public reference room, 100 F Street, N.E., Washington, D.C. 20549. Information regarding the operation of the public reference section can be obtained by calling 1-800-SEC-0330. The SEC also maintains an Internet site at <http://www.sec.gov> that contains reports, statements and other information about issuers, such as us, who file electronically with the SEC. We maintain a Internet sites at [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com) and [www.plasmatechbio.com](http://www.plasmatechbio.com). However, the information on our Internet sites is not incorporated by reference into this prospectus and any prospectus supplement and you should not consider it a part of this prospectus or any accompanying prospectus supplement.

The SEC allows us to "incorporate by reference" into this prospectus the information in other documents that we file with it. This means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be a part of this prospectus, and information in documents that we file later with the SEC will automatically update and supersede information contained in documents filed earlier with the SEC or contained in this prospectus. We incorporate by reference into this prospectus the documents listed below and any future filings that we may make with the SEC under Sections 13(a), 13(c), 14, or 15(d) of the Exchange Act prior to the termination of the offering under this prospectus; provided, however, that we are not incorporating, in each case, any documents or information deemed to have been furnished and not filed in accordance with SEC rules:

- Our Annual Report on Form 10-K for the year ended December 31, 2017 (filed on March 16, 2018) (including the information in Part III incorporated by reference from our Definitive Proxy Statement on Schedule 14A (filed on April 10, 2018);
- Our Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2018 (filed on May 10, 2018);
- Our Current Report on Form 8-K filed on February 9, 2018; and Current Report on Form 8-K filed on filed on April 2, 2018 (only as to the items that are filed and not furnished);
- Definitive Proxy Statement on Schedule 14A relating to the Company's 2018 Annual Meeting of Shareholders (filed on April 10, 2018); and
- The description of our common stock, par value \$0.01 per share contained in our Registration Statement on Form 8-A, dated and filed with the SEC on November 4, 2014, and any amendment or report filed with the SEC for the purpose of updating the description.

All reports and other documents we subsequently file pursuant to Section 13(a), 13(c), 14 or 15(d) of the Exchange Act prior to the termination of this offering, including all such documents we may file with the SEC after the date of this initial registration statement and prior to the effectiveness of this registration statement, but excluding any information furnished to, rather than filed with, the SEC, will also be incorporated by reference into this prospectus and deemed to be part of this prospectus from the date of the filing of such reports and documents.

You may obtain a copy of any or all of the documents referred to above which may have been or may be incorporated by reference into this prospectus, except for exhibits to those documents (unless the exhibits are specifically incorporated by reference into those documents) at no cost to you by writing or telephoning us at the following address: Investor Relations, Abeona Therapeutics Inc., 1330 Avenue of the Americas, 33<sup>rd</sup> Floor, New York, NY 10019, telephone (646) 813-4705.

### **LEGAL MATTERS**

Unless otherwise specified in the prospectus supplement accompanying this prospectus, Morgan, Lewis & Bockius LLP will provide opinions regarding certain legal matters. Certain partners and attorneys of Morgan, Lewis & Bockius LLP hold shares of our common stock. Additional legal matters may be passed upon for us or any underwriters, dealers or agents, by counsel that we will name in the applicable prospectus supplement.

### **EXPERTS**

The consolidated financial statements, incorporated by reference from the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2017, have been audited by Whitley Penn LLP, an independent registered public accounting firm, as stated in their report, which is incorporated by reference into this prospectus. Such financial statements have been so incorporated in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

**\$150,000,000**



**Common Stock**

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**PROSPECTUS SUPPLEMENT**

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**Jefferies LLC**

August 17, 2018

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