AMICUS THERAPEUTICS INC
Form 424B5
July 12, 2017
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Filed pursuant to Rule 424(b)(5)
Registration No.: 333-211005

The information in this preliminary prospectus supplement is not complete and may be changed. This preliminary prospectus supplement and the accompanying prospectus are not an offer to sell these securities, nor are they soliciting offers to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated July 12, 2017

Preliminary Prospectus Supplement
(To Prospectus dated April 29, 2016)

AMICUS THERAPEUTICS, INC.

$225,000,000

We are offering shares of our common stock, par value $0.01 per share, at an aggregate public offering price of up to $225,000,000.

Our common stock is listed on The NASDAQ Global Market under the symbol FOLD. On July 11, 2017, the last reported sale price of our common stock on The NASDAQ Global Market was $12.92 per share.

Investing in our securities involves a high degree of risk. You should review carefully the risks and uncertainties described under the heading Risk Factors on page S-7 of this prospectus supplement, page 3 of the accompanying prospectus and under similar headings in the other documents that are incorporated by

reference in this prospectus supplement and the accompanying prospectus.

	Per share	Total
Public offering price	$	$
Underwriting discounts and commissions	$	$
Proceeds to us before expenses	$	$

The underwriters may also purchase up to an additional $33,750,000 of our common stock from us at the public offering price, less underwriting discounts and commissions, within 30 days of the date of this prospectus supplement. If the underwriters exercise this option in full, the total underwriting discounts and commissions will be $, and our total proceeds before expenses, will be $.

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

The underwriters expect to deliver the shares of our common stock on or about July , 2017.

J.P. Morgan **Goldman Sachs & Co. LLC**

The date of this prospectus supplement is July , 2017.

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Prospectus Supplement

Prospectus

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About This Prospectus Supplement

This prospectus supplement and the accompanying prospectus are part of a universal shelf registration statement on Form S-3 (File No. 333-211005) that we filed with the U.S. Securities and Exchange Commission (the SEC) on April 29, 2016, which became effective automatically upon the filing thereof. This document is in two parts. The first part is this prospectus supplement which describes the terms of this offering of our common stock and adds to and updates the information contained in the accompanying prospectus. The second part, 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 both parts of this document combined. To the extent there is a conflict between the information contained in this prospectus supplement and the information contained in the accompanying prospectus or in any document incorporated by reference into this prospectus supplement that was filed with the SEC before the date of this prospectus supplement, you should rely on the information in this prospectus supplement.

This prospectus supplement and the accompanying prospectus relate to the offering of shares of our common stock. Before buying any of the shares of common stock offered hereby, we urge you to read carefully this prospectus supplement and the accompanying prospectus, together with the information incorporated herein by reference as described below under the heading Incorporation of Certain Information by Reference. This prospectus supplement contains information about the common stock offered hereby and may add to, update or change information in the accompanying prospectus.

You should rely only on the information contained in, or incorporated by reference into, this prospectus supplement and the accompanying prospectus. We have not, and the underwriters have not, authorized anyone to provide you with different or additional information.

We are not making offers to sell or solicitations 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. Persons outside the United States who come into possession of this prospectus supplement and accompanying prospectus must inform themselves about, and observe any restrictions relating to, the offering of our securities and the distribution of this prospectus supplement and accompanying prospectus outside the United States. You should assume that the information in this prospectus supplement and the accompanying prospectus is accurate only as of the date on the front of the respective document and that any information that we have incorporated by reference is accurate only as of the date of the document incorporated by reference, regardless of the time of delivery of this prospectus supplement or the accompanying prospectus or the time of any sale of a security.

This prospectus supplement and the accompanying prospectus contain summaries of certain provisions contained in some of the documents described herein, but reference is made to the actual documents for complete information. All of the summaries are qualified in their entirety by the actual documents. Copies of some of the documents referred to herein have been filed, will be filed or will be incorporated herein by reference as exhibits to the registration statement, and you may obtain copies of those documents as described below under the section entitled Incorporation of Certain Information by Reference.

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

This prospectus supplement and the accompanying prospectus contain and incorporate by reference market data and industry statistics and forecasts that are based on independent industry publications and other publicly-available information. Although we believe these sources are reliable, we do not guarantee the accuracy or completeness of this information and we have not independently verified this information.

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Although we are not aware of any misstatements regarding the market and industry data presented in this prospectus supplement, accompanying prospectus or the documents incorporated herein by reference, these estimates involve risks and uncertainties and are subject to change based on various factors. Accordingly, investors should not place undue reliance on this information.

Unless the context otherwise requires, in this prospectus supplement the Company, we, us, our and similar names refer to Amicus Therapeutics, Inc., a Delaware corporation, and its consolidated subsidiary.

This prospectus supplement and the accompanying prospectus and the information incorporated herein by reference include trademarks, service marks and trade names owned by us or other companies. We have filed applications to register certain trademarks in the U.S. and abroad, including Amicus Therapeutics® and designs, At the forefront of therapies for rare and orphan diseases , Zorblisa , and Galafold . All other trademarks or trade names referred to in this prospectus are the property of their respective owners.

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Prospectus Supplement Summary

This summary highlights selected information about us and this offering and does not contain all of the information that you should consider in making your investment decision. You should carefully read this entire prospectus supplement and the accompanying prospectus, including the risks and uncertainties discussed under the heading Risk Factors beginning on page S-7 of this prospectus supplement, and the information incorporated by reference in this prospectus supplement, including our financial statements, before making an investment decision. If you invest in our securities, you are assuming a high degree of risk.

Our Company

Overview

We are a global patient-focused biotechnology company engaged in the discovery, development and commercialization of a diverse set of novel treatments for patients living with devastating rare and orphan diseases. Our lead product, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease. Migalastat was approved for use in the EU in May 2016 under the brand name Galafold as a first-line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. The approved label includes 313 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. As of June 30, 2017, we have been authorized to commercialize Galafold™ in 33 countries and over 150 Fabry patients receiving Galafold™ compared to approximately 100 in April 2017. We remain confident in our guidance to achieve over 300 patients on Galafold™ therapy by the end of 2017. Additionally, based on a series of discussions with and written communication received from the FDA, the FDA has informed us that we may now submit an NDA for migalastat. An additional Phase 3 study previously requested by the FDA to assess Gastrointestinal (GI) symptoms is no longer required to support an NDA submission. We are preparing the NDA submission under Subpart H, which provides for accelerated approval. We plan to submit an NDA to the FDA for migalastat for Fabry disease in the fourth quarter of 2017.

Also in the pipeline, SD-101 is a product candidate in late-stage development, as a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa (EB). We are also leveraging our Chaperone-Advanced Replacement Therapy (CHART) platform technologies to develop novel ERT products for Pompe disease, Fabry disease, and potentially other lysosomal storage disorders (LSDs). We are also investigating preclinical and discovery programs in other rare and devastating diseases including cyclin-dependent kinase-like 5 (CDKL5) deficiency. We believe that our platform technologies and our product pipeline uniquely position us at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

Our Strategy

Our strategy is to internally develop or acquire first-in-class or potentially best-in-class therapies that have the potential to provide significant benefits for individuals living with rare and devastating diseases. We intend to leverage our global capabilities to develop and commercialize our robust pipeline. Since the beginning of our last fiscal year, we made significant progress toward fulfilling our vision to build a leading global biotechnology company focused on rare and devastating diseases:

- *Global capabilities*. We have established a world-class international commercial infrastructure, with key leadership in place to execute the international launch of migalastat HCl that is currently underway.

- *Commercial success.* We received full approval in the European Union (EU) of migalastat HCI under the brand name Galafold and commenced the first commercial launch in Germany on May 30, 2016. We have achieved success with reimbursement in 12 EU member states and other parts of the world on a commercial basis or through our expanded access programs (EAPs). We continue to advance additional regulatory submissions in multiple countries around the world.

- *NDA Submission under Subpart H*. We are preparing a New Drug Application (NDA) submission under Subpart H, which provides for accelerated approval by the U.S. Food and Drug Administration (FDA), for migalastat HCl for Fabry Disease. We intend to base our NDA on existing data, including reduction in disease-causing substrate (GL-3), as well as the totality of data from completed clinical studies. We plan to submit our NDA in the fourth quarter of 2017.

- *Pompe clinical study.* We have reported positive preliminary data from a clinical study to evaluate Pompe disease patients treated with our novel treatment paradigm ATB200/AT2221.

- *Late-stage product development.* We continue to investigate SD-101, a proprietary topical medicine for all major types of EB, in a single Phase 3 registration study that we hope will support global applications for approval in a number of countries. SD-101 has been granted Breakthrough Therapy designation by the FDA.

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- *Patient-centricity.* We continue to focus on our patient advocacy, which has always been a critical component of the values of our corporate culture, throughout all levels of the organization. The needs of patients in the rare disease community are at the center of our inventive science, our commercial organization, and our clinical programs.

Our Commercial Product and Product Candidates

Migalastat for Fabry Disease

Our Fabry franchise strategy is to develop migalastat HCl (which we may refer to as migalastat) for all patients with Fabry disease as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients. Migalastat was approved for use in the EU in May 2016 under the brand name Galafold as a first-line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. The approved label includes 313 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. Outside of the EU and Switzerland, migalastat is an investigational product.

We have launched Galafold in several European markets, including countries such as France, Germany, Italy, Switzerland and the UK, on a commercial basis, as well as in select other European markets through reimbursed EAPs, and recognized net product sales of $4.2 million in the three months ended March 31, 2017 as compared to $2.8 million in the fourth quarter of 2016. We are currently pursuing the country-by-country pricing and reimbursement process in the EU member states. We have received marketing approval in Israel, and we have regulatory submissions under review in additional countries, including Japan, Canada and Australia.

Based on a series of discussions with and written communication received from the FDA, the FDA has informed us that we may now submit an NDA for migalastat. An additional Phase 3 study previously requested by the FDA to assess Gastrointestinal (GI) symptoms is no longer required to support an NDA submission. We are preparing the NDA submission under Subpart H, which provides for accelerated approval. We intend to base our NDA on existing data, including reduction in disease-causing substrate (GL-3), as well as the totality of data from completed clinical studies. Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease and stroke. We plan to submit an NDA to the FDA for migalastat for Fabry disease in the fourth quarter of 2017.

For patients with non-amenable mutations, we are leveraging our CHART technology and advanced biologics capabilities to develop a proprietary Fabry ERT for co-formulation with migalastat. Master cell banking has been completed and process development work has commenced. Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations, and at this time, it is not intended for concomitant use with ERT.

SD-101 for EB

We are in Phase 3 development of a novel, late-stage, proprietary topical medicine, SD-101, a potentially first-to-market therapy for the treatment of skin blistering and lesions associated with all major types of EB. ESSENCE, a Phase 3 registration-directed study, was initiated in March of 2015 and completed enrollment of more than 160 patients in April of 2017. ESSENCE is a randomized, double-blind, placebo-controlled study being conducted at multiple sites worldwide that is designed to evaluate the safety and efficacy of SD-101 6% in patients with any of the three major types of EB who are at least one-month old. Participants are being randomized 1:1 to two treatment groups receiving either SD-101 6% or placebo applied over their entire body once daily for three months.

SD-101 was one of the first therapies to receive Breakthrough Therapy designation by the FDA in 2013, following the completion of the Phase 2a initial human proof-of-concept study. The FDA and EMA each have also reviewed the Phase 2b study results. The FDA agreed to a rolling NDA in the United States, which was initiated in the fourth quarter of 2015. Following the Phase 2b study, the Paediatric Committee of the EMA has issued a positive opinion on our Paediatric Investigation Plan (PIP) for SD-101. A PIP is part of the

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EMA approval process and must be accepted prior to a submission of a marketing authorisation application (MAA) in the EU. Results from the Phase 3 study are anticipated in mid-2017 to support marketing applications for SD-101 in the United States, EU, and other regions.

Novel ERT for Pompe Disease

We are leveraging our biologics capabilities and CHART platform to develop a novel treatment paradigm, ATB200/AT2221, for Pompe disease. This ERT consists of a uniquely engineered recombinant human acid alpha-glucosidase enzyme, ATB200, with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone (AT2221) to improve activity and stability. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma.

The small molecule pharmacological chaperone AT2221 is not an active ingredient that contributes directly to GAA substrate reduction but instead acts to stabilize ATB200. AT2221 binds and stabilizes ATB200 in the circulation to improve the uptake of active enzyme into key disease-relevant tissues, resulting in increased clearance of accumulated substrate glycogen. The novel combination has been patented for method of use, and ATB200, following significant manufacturing scale-up, is our first biologic to enter clinical development. In preclinical studies, administration of ATB200/AT2221 resulted in decreased tissue GAA enzyme levels and substrate reduction.

A Phase 1/2 clinical study, ATB200-02, was initiated in December of 2015 to investigate our novel Pompe treatment paradigm in Pompe patients. The primary objective is to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 for an 18-week primary treatment period followed by a long-term extension. The three patient cohorts, enrolling up to ~20 total patients across all cohorts, are ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Reported preliminary data on May 15, 2017 highlights include safety data for 20 patients (maximum 48 weeks) as well as PD (muscle biomarker and disease substrate biomarker) data for 16 patients (11 ERT-switch patients and five ERT-naïve patients). ATB200/AT2221 safety measures showed no serious adverse events (SAEs) considered related to ATB200/AT2221 with TEAEs that were generally mild and transient. To date, ATB200/AT2221 has shown no infusion-associated reactions following 200+ infusions. The clinical PK profile was consistent with previously reported preclinical data. Reductions were observed in biomarkers of muscle damage (creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in a majority of ERT-switch patients and ERT-naïve patients, and across the three biomarkers, mean reductions from baseline were approximately 15-20% and 50-55% for the ERT-switch and ERT-naïve patients, respectively. Reduction was also observed in a biomarker of glycogen substrate - Urine Hexose Tetrasaccharide (Hex4) - in a majority of ERT-switch patients and all ERT-naïve patients, with mean reductions from baseline of approximately 40% and 50% for the ERT-switch and ERT-naïve patients, respectively.

Functional outcomes data from baseline to Month 6 are currently available for 10 patients (seven ambulatory ERT-switch, two ERT-naïve and one non-ambulatory ERT-switch). Muscle function improved in 9/10 patients. Mean six minute walk test (6MWT) distance improved in both ERT-naive (+52 Meters) and ERT-switch (+38 Meters) patients (8 out of 9). Other motor function tests in ambulatory patients were consistent with the 6MWT. First non-ambulatory patient showed significant improvements in muscle strength tests. Pulmonary function at Month 6 showed forced vital capacity (FVC) increased in ERT-naive patients (mean +3.0%) and was stable in ERT-switch patients (mean +0.3%). Maximal inspiratory pressure (MIP) and maximal expiratory pressure (MEP) were generally consistent with FVC.

CDKL5

We are researching a potential first-in-class protein replacement therapy approach for CDKL5 deficiency in preclinical studies. CDKL5 is a gene on the X-chromosome encoding the CDKL5 protein that regulates the expression of several essential proteins for normal brain development. Genetic mutations in the CDKL5 gene result in CDKL5 protein deficiency and the disorder manifests clinically as persistent seizures starting in infancy, followed by severe impairment in neurological development. Most children affected by CDKL5 deficiency cannot walk or care for themselves and may also suffer from scoliosis, visual impairment, sensory issues, and gastrointestinal complications.

Strategic Alliances and Arrangements

We will continue to evaluate business development opportunities as appropriate that build stockholder value and provide us with access to the financial, technical, clinical, and commercial resources

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necessary to develop and market pharmacological chaperone therapeutics, ERTs, skin treatments, and other technologies or products. We are exploring potential collaborations, alliances, and other business development opportunities on a regular basis. These opportunities may include the acquisition of preclinical-stage, clinical-stage or marketed products so long as such transactions are consistent with our strategic plan to develop and provide therapies to patients living with rare and orphan diseases, and support our continued transformation from a development-stage company into a commercial biotechnology company.

Corporate information

We were incorporated under the laws of the State of Delaware on February 4, 2002. Our principal executive offices are located at 1 Cedar Brook Drive, Cranbury, NJ 08512 and our telephone number is (609) 662-2000. Our website address is *www.amicusrx.com*. We make available free of charge on our website our annual, quarterly and current reports, including amendments to such reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the SEC. Information contained on our website is not incorporated by reference into this prospectus supplement or the accompanying prospectus, and you should not consider information contained on our website as part of this prospectus supplement or the accompanying prospectus.

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The Offering

Common stock offered by us pursuant to this prospectus supplement	Shares having an aggregate offering price of up to $225,000,000.
Option to purchase additional shares	We have granted the underwriters an option for a period of up to 30 days from the date of this prospectus supplement to purchase up to an additional $33,750,000 of our common stock at the public offering price less the underwriting discounts and commissions.
Common stock to be outstanding immediately after this offering	shares (shares assuming the underwriters exercise in full their option to purchase additional shares).
Use of Proceeds	We currently intend to use the net proceeds of this offering for investment in the U.S. and international commercial infrastructure for migalastat HCl, investment in manufacturing capabilities for ATB200, the continued clinical development of our product candidates, research and development expenditures, clinical and preclinical trial expenditures, commercialization expenditures and for other general corporate purposes, which may include working capital, capital expenditures, the funding of in-licensing agreements for product candidates, additional technologies or other forms of intellectual property, the acquisition of assets or businesses that are complementary to our existing business and general and administrative expenses. See Use of Proceeds on page S-11 of this prospectus supplement.
Risk Factors	An investment in our common stock involves a high degree of risk. See the information contained in or incorporated by reference under Risk Factors on page S-7 of this prospectus supplement, page 3 of the accompanying prospectus, page 34 of our Annual Report on Form 10-K for the year ended December 31, 2016, as amended, and under similar headings in the other documents that are incorporated by reference herein, as well as the other information included in or incorporated by reference in this

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	prospectus supplement and the accompanying prospectus.
Market for the common stock	Our common stock is quoted and traded on The NASDAQ Global Market under the symbol FOLD.

The number of shares of our common stock to be outstanding immediately after this offering is based on 142,829,530 shares of common stock outstanding as of March 31, 2017. Unless specifically stated otherwise, the information in this prospectus supplement is as of March 31, 2017 and excludes:

- 17,821,788 shares of our common stock issuable upon the exercise of stock options outstanding as of March 31, 2017, at a weighted average exercise price of $7.05 per share, of which options to purchase 8,563,990 shares of our common stock were then exercisable;
- 3,110,000 shares of our common stock issuable upon the exercise of warrants to purchase common stock, at a weighted-average exercise price of $7.59 per share;
- 3,021,857 shares of our common stock issuable upon the vesting of restricted stock units outstanding as of March 31, 2017;
- an aggregate of 7,721,595 shares of our common stock reserved for future grants of stock options (or other similar equity instruments) under the Amended and Restated Equity Incentive Plan; and
- 40,849,675 shares of common stock issuable upon conversion of 3.00% Convertible Senior Notes due 2023; and
- 547,089 shares of our common stock issued since March 31, 2017 upon the exercise of outstanding stock options or vesting of restricted stock units.

Except as otherwise indicated, all information in this prospectus supplement assumes no exercise by the underwriters of their option to purchase additional shares of common stock.

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Risk Factors

Investing in our common stock involves a high degree of risk. Before investing in our common stock, you should carefully consider the risks described below, together with all of the other information contained in this prospectus supplement and the accompanying prospectus and incorporated by reference herein and therein, including from our most recent Annual Report on Form 10-K, as amended, and subsequent Quarterly Reports on Form 10-Q. Some of these factors relate principally to our business and the industry in which we operate. Other factors relate principally to your investment in our securities. The risks and uncertainties described therein and below are not the only risks facing us. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also materially and adversely affect our business and operations.

If any of the matters included in the following risks were to occur, our business, financial condition, results of operations, cash flows or prospects could be materially and adversely affected. In such case, you may lose all or part of your investment.

Risks related to this offering

The exercise of options and warrants and other issuances of shares of common stock or securities convertible into or exercisable for shares of common stock following this offering will dilute your ownership interests and may adversely affect the future market price of our common stock.

Sales of our common stock in the public market, either by us or by our current stockholders, or the perception that these sales could occur, could cause a decline in the market price of our securities. All of the shares of our common stock held by those of our current stockholders who have not entered into lock-up agreements with the underwriters may be immediately eligible for resale in the open market either in compliance with an exemption under Rule 144 promulgated under the Securities Act of 1933, as amended (the Securities Act), or pursuant to an effective resale registration statement that we have previously filed with the SEC. Such sales, along with any other market transactions, could adversely affect the market price of our common stock.

In addition, as of March 31, 2017, there were outstanding options to purchase an aggregate of 17,821,788 shares of our common stock at a weighted average exercise price of $7.05 per share, of which options to purchase 8,563,990 shares of our common stock were then exercisable and 3,021,857 shares of our common stock issuable upon the vesting of restricted stock units outstanding as of March 31, 2017. As of March 31, 2017, there were warrants outstanding to purchase 3,110,000 shares of our common stock, with a weighted-average exercise price of $7.59 per share, and 40,849,675 shares of common stock issuable upon conversion of 3.00% Convertible Senior Notes due 2023. The exercise of options and warrants or conversion of notes at prices below the market price of our common stock could adversely affect the price of shares of our common stock. Additional dilution may result from the issuance of shares of our common stock in connection with collaborations or manufacturing arrangements or in connection with other financing efforts.

Any issuance of our common stock that is not made solely to then-existing stockholders proportionate to their interests, such as in the case of a stock dividend or stock split, will result in dilution to each stockholder by reducing his, her or its percentage ownership of the total outstanding shares. Moreover, if we issue options or warrants to purchase our common stock or notes convertible into our common stock in the future and those options or warrants are exercised or convertible notes are converted, you may experience further dilution. Holders of shares of our

common stock have no preemptive rights that entitle them to purchase their pro rata share of any offering of shares of any class or series.

You will suffer immediate and substantial dilution in the securities you purchase.

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The public offering price of $ per share of our common stock is substantially higher than the pro forma net tangible book value per share of our outstanding shares immediately after this offering. As a result, investors purchasing securities in this offering will incur immediate and substantial dilution of approximately $ per share of common stock, or approximately % of the public offering price. Accordingly, existing stockholders will benefit disproportionately from this offering. If we raise additional capital through the sale of equity, including convertible securities, your percentage of ownership will be diluted. You may also experience additional dilution if stock options or warrants to purchase our shares are exercised or convertible notes are converted at less than the offering price. As of March 31, 2017, we had reserved 7,721,595 shares of our common stock for issuance under our Amended and Restated Equity Incentive Plan, 40,849,675 shares of our common stock for issuance upon conversion of our 3.00% Convertible Senior Notes due 2023 and 3,110,000 shares of our common stock for issuance upon exercise of outstanding warrants.

We have broad discretion in the use of the net proceeds of this offering and, despite our efforts, we may use the proceeds in a manner that does not increase the value of your investment.

We currently anticipate that the net proceeds from the sale of our common stock will be used for investment in the international commercial infrastructure for migalastat HCl, the continued clinical development of our product candidates, research and development expenditures, clinical and preclinical trial expenditures, commercialization expenditures and for other general corporate purposes, which may include working capital, capital expenditures, the funding of in-licensing agreements for product candidates, additional technologies or other forms of intellectual property, the acquisition of assets or businesses that are complementary to our existing business and general and administrative expenses. However, we have not determined the specific allocation of the net proceeds among these potential uses. Our management will have broad discretion over the use and investment of the net proceeds of this offering, and, accordingly, investors in this offering will need to rely upon the judgment of our management with respect to the use of proceeds, with only limited information concerning our specific intentions. These proceeds could be applied in ways that do not improve our operating results or increase the value of your investment. Please see the section entitled Use of Proceeds on page S-11 of this prospectus supplement for further information.

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Special Note Regarding Forward-Looking Statements

This prospectus supplement, the accompanying prospectus and the documents incorporated by reference herein and therein contain forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this prospectus supplement, the accompanying prospectus and the documents incorporated by reference herein and therein regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words anticipate, believe, estimate, expect, potential, intend, may, plan, predict, project, w and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus supplement, the accompanying prospectus and the documents incorporated by reference herein and therein include, among other things, statements about:

- our expectations related to the use of proceeds, if any, from this offering;
- the progress and results of our clinical trials of our drug candidates;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new Fabry ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of LSDs;
- the future results of on-going or subsequent clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and obtain market acceptance of SD-101;
- the future results of on-going preclinical research and subsequent clinical trials for CDKL5, including our ability to obtain regulatory approvals and commercialize CDKL5 and obtain market acceptance for CDKL5;
- the costs, timing and outcome of regulatory review of our product candidates;

- the number and development requirements of other product candidates that we pursue;

- the costs of commercialization activities, including product marketing, sales and distribution;

- the emergence of competing technologies and other adverse market developments;

- our ability to obtain reimbursement for migalastat HCI;

- our ability to obtain market acceptance of migalastat HCl in the EU;

- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property-related claims;

- the extent to which we acquire or invest in businesses, products and technologies;

- our ability to successfully integrate our acquisitions of Scioderm, Inc. and MiaMed, Inc. and their products and technologies into our business, including the possibility that the expected benefits of the transactions will not be fully realized by us or may take longer to realize than expected; and

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- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

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 under Risk Factors 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 herein and therein completely and with the understanding that our actual future results may be materially different from what we expect.

Except as required by law, we undertake no obligation to update or revise any forward-looking statements to reflect new information or future events or developments. You should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. Before deciding to purchase our securities, you should carefully consider the risk factors discussed and incorporated by reference in this prospectus supplement and the accompanying prospectus and in the registration statement of which this prospectus supplement and the accompanying prospectus form a part.

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Use of Proceeds

We expect to receive net proceeds of approximately $ from the sale of shares of our common stock in this offering, or $ if the underwriters exercise in full their option to purchase additional shares of common stock, based on a public offering price of $ per share after deducting the estimated expenses related to this offering and the underwriting discounts and commissions payable by us.

We currently intend to use the net proceeds from the sale of the shares of common stock offered by us hereunder for, without limitation:

- investment in the U.S. and international commercial infrastructure for migalastat HCl;
- investment in manufacturing capabilities for ATB200;
- the continued clinical development of our product candidates;
- research and development expenditures;
- clinical and preclinical trial expenditures;
- commercialization expenditures; and
- for other general corporate purposes, which may include working capital, capital expenditures, the funding of in-licensing agreements for product candidates, additional technologies or other forms of intellectual property, the acquisition of assets or businesses that are complementary to our existing business and general and administrative expenses.

The amounts and timing of our use of the net proceeds from this offering will depend on a number of factors, such as the timing and progress of our research and development efforts, the timing and progress of any partnering and commercialization 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 the sale of the shares of common stock offered by us hereunder. 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 intend to temporarily invest the proceeds in short-term, interest-bearing instruments.

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Dilution

Purchasers of common stock in this offering will experience immediate dilution to the extent of the difference between the public offering price per share of common stock, and the net tangible book value per share of common stock immediately after this offering.

Our net tangible book value as of March 31, 2017 was approximately $(374) million, or $(2.62) per share of common stock. Net tangible book value per share is determined by dividing total tangible assets less total liabilities by the aggregate number of shares of common stock outstanding a