

AMICUS THERAPEUTICS INC

Form 10-Q

November 07, 2016

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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-Q**

(Mark One)

**QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2016

OR

**TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number 001-33497

## Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation or Organization)

**71-0869350**  
(I.R.S. Employer  
Identification Number)

**1 Cedar Brook Drive, Cranbury, NJ 08512**  
(Address of Principal Executive Offices and Zip Code)

Registrant's Telephone Number, Including Area Code: **(609) 662-2000**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days: Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller-reporting company. See definition of large accelerated filer, accelerated filer and smaller-reporting company in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer   
Non-accelerated filer

Accelerated filer   
Smaller Reporting Company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act): Yes  No

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of October 28, 2016 was 142,326,195 shares.

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AMICUS THERAPEUTICS, INC.

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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 , Galafold .

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**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This quarterly report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this quarterly report on Form 10-Q 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, will, should, would and similar expressions are used in forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this quarterly report on Form 10-Q include, among other things, statements about:

- 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 enzyme replacement therapy ( 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 lysosomal storage disorders ( LSDs );
- the future results of on-going or later 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 and later clinical trials for cyclin-dependent kinase-like 5 ( 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 HCl;
- our ability to obtain market acceptance of migalastat HCl in the European Union (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 recent acquisitions of Scioderm, Inc. ( Scioderm ) and MiaMed, Inc. ( MiaMed ) 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
- 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 Part I Item 1A Risk Factors of the Annual Report on Form 10-K for the year ended December 31, 2015, 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 quarterly report on Form 10-Q in conjunction with the document that we reference herein. We do not assume any obligation to update any forward-looking statements.

Table of Contents**PART I. FINANCIAL INFORMATION****Item 1. Financial Statements (unaudited)****Amicus Therapeutics, Inc.****Consolidated Balance Sheets (Unaudited)**

(in thousands, except share and per share amounts)

	September 30, 2016	December 31, 2015
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 33,115	\$ 69,485
Investments in marketable securities	179,284	144,548
Accounts receivable	864	
Inventories	3,251	
Prepaid expenses and other current assets	5,198	2,568
Total current assets	221,712	216,601
Property and equipment, less accumulated depreciation of \$15,181 and \$13,353 at September 30, 2016 and December 31, 2015, respectively	10,183	6,178
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	1,788	1,108
<b>Total Assets</b>	<b>\$ 918,180</b>	<b>\$ 908,384</b>
<b>Liabilities and Stockholders Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 29,013	\$ 32,216
Contingent consideration payable, current portion	55,992	41,400
Other current liabilities	607	
Total current liabilities	85,612	73,616
Deferred reimbursements	35,756	35,756
Due to related party	44,047	41,601
Unsecured notes payable	21,977	
Contingent consideration payable, less current portion	216,198	232,677
Deferred tax liability	176,219	176,219
Other non-current liabilities	1,816	681
Commitments and contingencies		
Stockholders equity:		
Common stock, \$.01 par value, 250,000,000 authorized, 142,273,085 shares issued and outstanding at September 30, 2016, 250,000,000 shares authorized, 125,027,034 shares issued and outstanding at December 31, 2015	1,478	1,306
Additional paid-in capital	1,038,613	917,454

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Accumulated other comprehensive loss:

Foreign currency translation adjustment, less tax benefit of \$706 at September 30, 2016	1,062	
Unrealized gain/ (loss) on available-for securities	287	(115)
Warrants	16,076	8,755
Accumulated deficit	(720,961)	(579,566)
Total stockholders' equity	336,555	347,834
<b>Total Liabilities and Stockholders' Equity</b>	\$ 918,180	\$ 908,384

*See accompanying notes to consolidated financial statements*



Table of Contents**Amicus Therapeutics, Inc.****Consolidated Statements of Operations***(Unaudited)***(in thousands, except share and per share amounts)**

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Net product sales	\$ 2,127	\$	\$ 2,127	\$
Cost of goods sold	344		344	
Gross profit	1,783		1,783	
Operating Expenses:				
Research and development	32,457	20,971	74,163	54,318
Selling, general and administrative	17,469	15,372	52,470	30,077
Changes in fair value of contingent consideration payable	(4,110)	1,300	9,228	2,400
Restructuring charges	11	7	69	44
Loss on extinguishment of debt				952
Depreciation	896	395	2,336	1,256
Total operating expenses	46,723	38,045	138,266	89,047
Loss from operations	(44,940)	(38,045)	(136,483)	(89,047)
Other income (expenses):				
Interest income	460	316	1,098	645
Interest expense	(1,517)	(17)	(3,517)	(727)
Other expense	(910)	(54)	(3,199)	(93)
Loss before income tax benefit	(46,907)	(37,800)	(142,101)	(89,222)
Income tax benefit	253		706	
Net loss	(46,654)	(37,800)	(141,395)	(89,222)
Net loss per common share basic and diluted	\$ (0.33)	\$ (0.32)	\$ (1.07)	\$ (0.85)
Weighted-average common shares outstanding basic and diluted	140,656,109	118,724,882	131,675,690	104,885,956

*See accompanying notes to consolidated financial statements*

Table of Contents**Amicus Therapeutics, Inc.****Consolidated Statements of Comprehensive Loss***(Unaudited)***(in thousands)**

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2016</b>	<b>2015</b>	<b>2016</b>	<b>2015</b>
Net loss	\$ (46,654)	\$ (37,800)	\$ (141,395)	\$ (89,222)
Other comprehensive gain /(loss)				
Foreign currency translation adjustment, net of tax \$253 and \$706, respectively	220		1,062	
Unrealized gain/(loss) on available- for-sale securities	86	(91)	402	(10)
Other comprehensive gain /(loss)	\$ 306	\$ (91)	\$ 1,464	\$ (10)
Comprehensive loss	\$ (46,348)	\$ (37,891)	\$ (139,931)	\$ (89,232)

*See accompanying notes to consolidated financial statements*

Table of Contents**Amicus Therapeutics, Inc.****Consolidated Statements of Cash Flows***(Unaudited)***(in thousands)**

	<b>Nine Months Ended September 30,</b>	
	<b>2016</b>	<b>2015</b>
<b>Operating activities</b>		
Net loss	\$ (141,395)	\$ (89,222)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	1,744	136
Depreciation	2,336	1,256
Stock-based compensation	13,087	6,929
Charges to research expense for stock issued in asset acquisition	4,607	
Restructuring charges	69	44
Loss on extinguishment of debt		952
Loss on disposal of asset	17	
Non-cash changes in the fair value of derivative liability	324	
Non-cash changes in the fair value of contingent consideration payable	9,228	2,400
Foreign currency remeasurement loss	2,207	
Non-cash income tax benefit	(706)	
Changes in operating assets and liabilities:		
Accounts receivable	(863)	
Inventories	(3,505)	
Prepaid expenses and other current assets	(2,803)	(668)
Other non-current assets	(660)	(540)
Accounts payable and accrued expenses	(2,920)	14,118
Non-current liabilities	684	(33)
Net cash used in operating activities	(118,549)	(64,628)
<b>Investing activities</b>		
Sale and redemption of marketable securities	165,495	133,418
Purchases of marketable securities	(199,829)	(220,861)
Purchases of property and equipment	(5,520)	(2,246)
Acquisitions, net cash of acquired		(141,060)
Net cash used in investing activities	(39,854)	(230,749)
<b>Financing activities</b>		
Proceeds from issuance of common stock, net of issuance costs	97,068	243,042
Proceeds from unsecured note agreement	30,000	
Payments of secured loan agreement		(15,291)
Proceeds from related party		50,000
Payment of capital lease	(118)	
Payment of contingent consideration	(5,000)	
Proceeds from exercise of stock options	1,456	10,673
Purchase of vested restricted stock units	(1,010)	(1,682)
Proceeds from exercise of warrants		4,000
Net cash provided by financing activities	122,396	290,742
Effect of exchange rate changes on cash and cash equivalents	(363)	
Net decrease in cash and cash equivalents	(36,370)	(4,635)
Cash and cash equivalents at beginning of period	69,485	24,074

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Cash and cash equivalents at end of period	\$	33,115	\$	19,439
<b>Supplemental disclosures of cash flow information</b>				
Cash paid during the period for interest	\$	284	\$	605
Contingent consideration resolution in shares	\$	6,115	\$	
Capital expenditures funded by capital lease borrowings	\$	850	\$	

*See accompanying notes to consolidated financial statements*

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**Amicus Therapeutics, Inc.**

**Notes to the Consolidated Financial Statements**

*(Unaudited)*

**Note 1. Description of Business**

*Corporate Information, Status of Operations, and Management Plans*

Amicus Therapeutics, Inc. (the Company, we, us, or our ) is 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. The lead product, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement of therapy ( ERT ) for Fabry disease.

The Company's Fabry franchise strategy is to develop migalastat HCl (which the Company 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. In May 2016, the Company announced that the European Commission ( EC ) had granted full approval for the oral small molecule pharmacological chaperone Galafold (migalastat) 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 (alpha-galactosidase A deficiency) and who have an amenable mutation. The approved label includes 269 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. The Company commenced commercial shipments of Galafold in the EU in the second quarter of 2016 and recognized net product sales of \$2.1 million in the third quarter of 2016.

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 ). The Company is also leveraging its biologics and Chaperone-Advanced Replacement Therapy ( CHART ) platform technologies to develop novel ERT products for Pompe disease, Fabry disease, and potentially other lysosomal storage disorders ( LSDs ). The Company is also investigating preclinical and discovery programs in other rare and devastating diseases including CDKL5 deficiency. The Company believes that the platform technologies and advanced product pipeline uniquely position the Company at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

In July 2016, the Company expanded its biologics pipeline with a new preclinical program for CDKL5 deficiency, a rare and devastating genetic neurological disease for which there is no currently approved treatment. The Company has obtained the rights and related intellectual property to a preclinical CDKL5 program through its acquisition of MiaMed, Inc ( MiaMed ). Under the terms of the MiaMed Agreement and Plan of Merger (the MiaMed Agreement ), with MiaMed and certain other parties signatory thereto, in connection with the closing of the transactions contemplated by the MiaMed Agreement, the former holders of MiaMed's capital stock (collectively, the MiaMed Stockholders ) received an aggregate of \$6.5 million, comprised of (i) approximately \$1.8 million in cash (plus MiaMed's cash and cash equivalents at closing and less any of MiaMed's unpaid third-party fees and expenses related to the transaction), and (ii) 825,603 shares of Amicus common stock. In addition, Amicus also agreed to pay up to an additional \$83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of \$89.5 million.

On June 30, 2016, the Company entered into a Joinder to and Amendment of Note and Warrant Purchase Agreement (the Amended Purchase Agreement ) with Redmile Capital Fund, LP and certain of its affiliates (collectively referred to as Redmile ). Such amendment joined Grosvenor Special Opportunities Master Fund, Ltd. ( GCM ) to the Note and Warrant Purchase Agreement, dated as of February 19, 2016. At closing, the Company sold (a) \$30.0 million principal amount of additional notes and (b) five-year warrants to purchase 42 shares of common stock of the Company, par value \$0.01 per share ( Common Stock ) for every \$1,000 of the principal amount of Additional Notes purchased by each Purchaser ( Additional Warrants ), for an aggregate of approximately 1.3 million shares of Common Stock issuable under the Additional Warrants. For additional information, see Note 7. Debt Instruments and Related Party Transactions.

Beginning in April 2016 and through July 2016, the Company sold 15.0 million shares of Common Stock under an at-the-market ( ATM ) equity program with Cowen and Company, LLC ( Cowen ) acting as sales agent. Cowen was compensated at a fixed commission rate up to 3.0%. The ATM sales agreement resulted in net proceeds of \$97.1 million, after Cowen s commission of \$2.7 million and other expenses of \$0.2 million. The Company has completed all sales under the ATM equity program.

The Company had an accumulated deficit of approximately \$721.0 million at September 30, 2016 and anticipates incurring losses through the fiscal year ending December 31, 2016 and beyond. The Company has funded its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from its initial public offering and

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subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. The Company commenced commercial shipments of Galafold in the EU in the second quarter of 2016 and recognized net product sales of \$2.1 million in the third quarter of 2016. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into the fourth quarter of 2017.

**Note 2. Summary of Significant Accounting Policies**

The consolidated financial statements include the accounts of Amicus Therapeutics, Inc. and its wholly-owned subsidiaries, after the elimination of intercompany transactions.

***Basis of Presentation***

The Company has prepared the accompanying unaudited consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ( U.S. GAAP ) for interim financial information and with the instructions to Form 10-Q and Article 10-01 of Regulations S-X. Accordingly, they do not include all of the information and disclosures required by generally accepted accounting principles for complete financial statements. In the opinion of management, the accompanying unaudited financial statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's interim financial information.

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2015. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K for the fiscal year ended December 31, 2015.

***Foreign Currency Transactions***

The functional currency for most of our foreign subsidiaries is their local currency. For our non-U.S. subsidiaries that transact in a functional currency other than the U.S. dollar, assets and liabilities are translated at current rates of exchange at the balance sheet date. Income and expense items are translated at the average foreign exchange rates for the period. Adjustments resulting from the translation of the financial statements of our foreign operations into U.S. dollars are excluded from the determination of net income and are recorded in accumulated other comprehensive income, a separate component of equity.

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016 the Company entered into one forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company does not designate this forward contract as a hedging instrument under applicable accounting guidance and, therefore, changes in fair value are recorded in the Consolidated Statements of Operations.

*Use of Estimates*

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from those estimates.

*Concentration of Credit Risk*

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents and marketable securities. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company invests its marketable securities in high-quality commercial financial instruments. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on cash and cash equivalents or its marketable securities.

The Company is subject to credit risk from its accounts receivable related to its product sales of Galafold. The majority of the Company's accounts receivable at September 30, 2016 have arisen from product sales in Germany. The Company will periodically assess the financial strength of its customers to establish allowances for anticipated losses, if any. For accounts receivable that have arisen from named patient sales, the payment terms are predetermined and the Company evaluates the creditworthiness of each customer on a regular basis. To date, the Company has not incurred any credit losses.



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***Significant Accounting Policies***

There have been no material changes to the Company's significant accounting policies during the nine months ended September 30, 2016, as compared to the significant accounting policies disclosed in Note 2 of the Consolidated Financial Statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2015. However, the following accounting policies are the most critical in fully understanding and evaluating the Company's financial condition and results of operations. Additionally, the Company added new policies on inventory and product sales in the current quarter.

***Revenue Recognition***

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when persuasive evidence an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collection of the amounts due are reasonably assured and the Company has no further performance obligations.

***Net Product Sales***

The Company's net product sales consist solely of sales of Galafold for the treatment of Fabry disease in the EU. The Company has recorded revenue on sales where Galafold is available either on a commercial basis or through a reimbursed early access program. Orders for Galafold are generally received from pharmacies and the ultimate payor is typically a government authority.

The Company records revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

***Collaboration Revenue***

In multiple element arrangements, revenue is allocated to each separate unit of accounting and each deliverable in an arrangement is evaluated to determine whether it represents separate units of accounting. A deliverable constitutes a separate unit of accounting when it has standalone value and there is no general right of return for the delivered elements. In instances when the aforementioned criteria are not met, the deliverable is combined with the undelivered elements and the allocation of the arrangement consideration and revenue recognition is determined for the combined unit as a single unit of accounting. Allocation of the consideration is determined at arrangement inception on the basis of each unit's relative selling price. In instances where there is determined to be a single unit of accounting, the total consideration is applied as revenue for the single unit of accounting and is recognized over the period of inception through the date where the last deliverable within the single unit of accounting is expected to be delivered.

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The Company's current revenue recognition policies provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, the Company allocates revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific objective evidence ( VSOE ) if available, (ii) third party evidence ( TPE ) if VSOE is not available, or (iii) best estimated selling price ( BESP ) if neither VSOE nor TPE is available. The Company would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The BESP would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

The Company also considers the impact of potential future payments it makes in its role as a vendor to its customers and evaluates if these potential future payments could be a reduction of revenue from that customer. If the potential future payments to the customer are:

- a payment for an identifiable benefit;
- the identifiable benefit is separable from the existing relationship between the Company and its customer;
- the identifiable benefit can be obtained from a party other than the customer; and
- the Company can reasonably estimate the fair value of the identifiable benefit

then the payments are accounted for separate from the revenue received from that customer. If, however, all these criteria are not satisfied, then the payments are treated as a reduction of revenue from that customer.

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If the Company determines that any potential future payments to its customers are to be considered as a reduction of revenue, it must evaluate if the total amount of revenue to be received under the arrangement is fixed and determinable. If the total amount of revenue is not fixed and determinable due to the uncertain nature of the potential future payments to the customer, then any customer payments cannot be recognized as revenue until the total arrangement consideration becomes fixed and determinable.

The reimbursements for research and development costs under collaboration agreements that meet the criteria for revenue recognition are included in Research Revenue and the costs associated with these reimbursable amounts are included in research and development expenses.

In order to determine the revenue recognition for contingent milestones, the Company evaluates the contingent milestones using the criteria as provided by the Financial Accounting Standards Boards ( FASB ) guidance on the milestone method of revenue recognition at the inception of a collaboration agreement. The criteria requires that (i) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from the Company s activities to achieve the milestone, (ii) the milestone be related to past performance, and (iii) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved.

***Inventories and Cost of Cost of Goods Sold***

Until regulatory approval of Galafold, the Company expensed all manufacturing costs as research and development expense. Upon regulatory approval, the Company began capitalizing costs related to the purchase and manufacture of Galafold.

Inventories are stated at the lower of cost or market determined by the first-in, first-out method. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity as well as product shelf-life. In evaluating the recoverability of inventories produced, the probability that revenue will be obtained from the future sale of the related inventory is considered and inventory value is written down for inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statements of operations.

Cost of goods sold includes the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, provisions for excess and obsolete inventory, as well as estimated royalties payable. A portion of the inventory available for sale was expensed as research and development costs prior to regulatory approval and as such the cost of goods sold and related gross margins are not necessarily indicative of future cost of goods sold and gross margin.

***Fair Value Measurements***

The Company records certain asset and liability balances under the fair value measurements as defined by the FASB guidance. Current FASB fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for

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considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that market participants assumptions would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which is typically based on an entity's own assumptions, as there is little, if any, related market activity. In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment, and considers factors specific to the asset or liability.

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***Contingent Liabilities***

On an ongoing basis, the Company may be involved in various claims, and legal proceedings. On a quarterly basis, the Company reviews the status of each significant matter and assesses its potential financial exposure. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount can be reasonably estimated, the Company will accrue a liability for the estimated loss. Because of uncertainties related to claims and litigation, accruals will be based on the Company's best estimates based on available information. On a periodic basis, as additional information becomes available, or based on specific events such as the outcome of litigation or settlement of claims, the Company may reassess the potential liability related to these matters and may revise these estimates, which could result in a material adverse adjustments to the Company's operating results.

***New Accounting Pronouncements***

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*. This Accounting Standards Update addresses the following eight specific cash flow issues including Debt prepayment or debt extinguishment costs, contingent consideration payments made after a business combination and separately identifiable cash flows and application of the predominance principle. The amendments in this ASU apply to all entities. The amendments in this Update are effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted, including adoption in an interim period. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In May 2016, the FASB issued ASU 2016-12, *Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients*. The amendments address narrow-scope improvements to the guidance on collectability, noncash consideration, and completed contracts at transition. Additionally, the amendments provide a practical expedient for contract modifications at transition and an accounting policy election related to the presentation of sales taxes and other similar taxes collected from customers. These amendments are effective at the same date that Topic 606 is effective. Topic 606 is effective for public entities for annual reporting periods beginning after December 15, 2017, including interim reporting periods therein (i.e., January 1, 2018, for a calendar year entity). This Accounting Standards Update is the final version of Proposed Accounting Standards Update 2015-230 *Revenue from Contracts with Customers (Topic 606) Narrow-Scope Improvements and Practical Expedients*, which has been deleted. The Company will adopt the new ASU on January 1, 2018. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In April 2016, the FASB issued ASU 2016-10, *Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing*. The amendments clarify the following two aspects of Topic 606: (a) identifying performance obligations; and (b) the licensing implementation guidance. The amendments do not change the core principle of the guidance in Topic 606. The effective date and transition requirements for the amendments are the same as the effective date and transition requirements in Topic 606. Public entities should apply the amendments for annual reporting periods beginning after December 15, 2017, including interim reporting periods therein (i.e., January 1, 2018, for a calendar year entity). Early application for public entities is permitted only as of annual reporting periods beginning after December 15, 2016, including interim reporting periods within that reporting period. 2018. The Company will adopt the new ASU on January 1, 2018. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In March 2016, the FASB issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting*. The amendments are intended to improve the accounting for employee share-based payments and affect all organizations that issue share-based payment awards to their employees. Several aspects of the accounting for share-based payment award transactions are

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simplified, including: (a) income tax consequences; (b) classification of awards as either equity or liabilities; and (c) classification on the statement of cash flows. For public companies, the amendments are effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. Early adoption is permitted for any organization in any interim or annual period. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In March 2016, the FASB issued ASU 2016-08, *Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations (Reporting Revenue Gross versus Net)*. The amendments relate to when another party, along with the entity, is involved in providing a good or service to a customer. Topic 606 *Revenue from Contracts with Customers* requires an entity to determine whether the nature of its promise is to provide that good or service to the customer (i.e., the entity is a principal) or to arrange for the good or service to be provided to the customer by the other party (i.e., the entity is an agent). The amendments are intended to improve the operability and understandability of the implementation guidance on principal versus agent considerations. The effective date and transition of these amendments is the same as the effective date and transition of ASU 2014-09, *Revenue from Contracts with Customers (Topic 606)*. Public entities should apply the amendments in ASU 2014-09 for annual reporting periods beginning after December 15, 2017, including interim reporting periods therein (i.e., January 1, 2018, for a calendar year entity). The Company will implement the new ASU in the first quarter of 2018. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

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In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*. This update requires the recognition of lease assets and lease liabilities on the balance sheet for all lease obligations and disclosing key information about leasing arrangements. This update requires the recognition of lease assets and lease liabilities by lessees for those leases classified as operating leases under previous generally accepted accounting principles. This update will be effective for the Company for all annual and interim periods beginning after December 15, 2018, including interim periods within those fiscal years. Early application is permitted for all public business entities and all nonpublic business entities upon issuance. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

**Note 3. Cash, Money Market Funds and Marketable Securities**

As of September 30, 2016, the Company held \$33.1 million in cash and cash equivalents and \$179.3 million in available-for-sale securities which are reported at fair value on the Company's balance sheet. Unrealized gains and losses are reported within accumulated other comprehensive income/ (loss) in the statements of comprehensive loss. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating. Investments that have original maturities or greater than 3 months but less than 1 year are classified as short-term and investments with maturities that are greater than 1 year are classified as long-term.

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016 the Company entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company does not designate these forward contracts as hedging instruments under applicable accounting guidance and, therefore, changes in fair value are recorded as other income (expense) in the Consolidated Statements of Operations, with the corresponding liability in current liabilities on the Consolidated Balance Sheet. For the three and nine months ended September 30, 2016, the Company recognized a gain of \$22 thousand and a loss of \$324 thousand related to the derivative instruments not designated as hedging instruments in other income (expense) in the Consolidated Statements of Operations and the corresponding liability of \$324 thousand is recorded as other current liability in the Consolidated Balance Sheets.

Cash and available-for-sale securities are all current unless mentioned otherwise and consisted of the following as of September 30, 2016 and December 31, 2015 (in thousands):

	Cost	As of September 30, 2016		Fair Value
		Unrealized Gain	Unrealized Loss	
Cash balances	\$ 33,115	\$	\$	\$ 33,115
Corporate debt securities	79,658	5	(45)	79,618
Commercial paper	98,326	327		98,653
Certificate of deposit	1,013			1,013

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	\$	212,112	\$	332	\$	(45)	\$	212,399
Included in cash and cash equivalents	\$	33,115	\$		\$		\$	33,115
Included in marketable securities	\$	178,997	\$	332	\$	(45)	\$	179,284
Total cash and marketable securities	\$	212,112	\$	332	\$	(45)	\$	212,399



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	As of December 31, 2015				Fair Value
	Cost	Unrealized Gain	Unrealized Loss		
Cash balances	\$ 69,485	\$	\$	\$	69,485
Corporate debt securities	118,627	1	(154)		118,474
Commercial paper	25,686	38			25,724
Certificate of deposit	350				350
	\$ 214,148	\$ 39	\$ (154)	\$	214,033
Included in cash and cash equivalents	\$ 69,485			\$	69,485
Included in marketable securities	144,663	39	(154)		144,548
Total cash and marketable securities	\$ 214,148	\$ 39	\$ (154)	\$	214,033

For the nine months ended September 30, 2016 and the year ended December 31, 2015, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of September 30, 2016 and December 31, 2015 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months and as such are recognized in other comprehensive gain/(loss). The fair value of these available for sale securities in unrealized loss positions was \$60.3 million and \$118.5 million as of September 30, 2016 and December 31, 2015, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income ( AOCI ) in the Statements of Comprehensive Loss.

**Note 4. Inventories**

Inventories consist of work in process and finished goods related to the manufacture of Galafold. The following table summarizes the components of inventories at September 30, 2016 (in thousands):

(Dollars in thousands)	September 30, 2016
Work-in-process	3,111
Finished goods	140
<b>Total inventories</b>	<b>\$ 3,251</b>

There were no inventories on-hand as of December 31, 2015. Inventory manufactured prior to commercialization was expensed to research and development. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity, as well as product shelf-life. In evaluating the recoverability of inventories produced, the Company considers the probability that revenue will be obtained from the future sale of the related inventory. Inventory becomes obsolete when it has aged past its shelf-life, cannot be recertified and is no longer usable or able to be sold, or the inventory has been damaged. In such instances, a full reserve is taken against such inventory. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statement of operations. There have been no write-downs of inventory from the time inventory was first capitalized.



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**Note 5. Acquisitions**

**Acquisition of MiaMed, Inc**

On July 5, 2016, the Company entered into an Agreement and Plan of Merger (the "MiaMed Agreement") with MiaMed, Inc., ("MiaMed"). MiaMed is a pre-clinical biotechnology company focused on developing protein replacement therapy for CDKL5 and related diseases. Under the terms of the MiaMed Agreement, the former holders of MiaMed's capital stock received an aggregate of \$6.5 million, comprised of (i) approximately \$1.8 million in cash (plus MiaMed's cash and cash equivalents at closing and less any of MiaMed's unpaid third-party fees and expenses related to the transaction), and (ii) 825,603 shares of the Company's Common Stock. In addition, the Company also agreed to pay up to an additional \$83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of \$89.5 million. The Company evaluated the transaction based on the guidance of ASC 805, *Business Combinations* and concluded that it only acquired inputs and did not acquire any processes. The Company will need to develop its own processes in order to produce an output. Therefore, the Company accounted the transaction as an asset acquisition and accordingly \$6.5 million was expensed to research and development.

**Acquisition of Scioderm, Inc.**

On September 30, 2015, the Company acquired Scioderm, a privately-held biopharmaceutical company focused on developing innovative therapies for treating the rare disease EB. The acquisition leverages the Scioderm development team's EB expertise with the Company's global clinical infrastructure to advance SD-101 toward regulatory approvals and the Company's commercial, patient advocacy, and medical affairs infrastructure to support a successful global launch. The acquisition of Scioderm was accounted for as a purchase of a business in accordance with FASB Accounting Standard Codification 805 *Business Combinations*.

The Company acquired Scioderm with cash and stock. At closing, the Company paid Scioderm stockholders, option holders, and warrant holders approximately \$223.9 million, of which approximately \$141.1 million was paid in cash and approximately \$82.8 million was paid through the issuance of approximately 5.9 million newly issued shares of the Company. The Company had agreed to pay up to an additional \$361 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain clinical and regulatory milestones, and \$257 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain sales milestones. If SD-101 is approved, EB qualifies as a rare pediatric disease under The Food and Drug Administration Safety and Innovation Act ("FDISA") and the Company will request a Priority Review Voucher ("PRV") under the FDISA, if available. If the PRV is obtained and subsequently sold, the Company will pay Scioderm stockholders, option holders, and warrant holders the lesser of \$100 million in the aggregate or 50% of the proceeds of such sale. If the Company obtains the PRV and has not entered into an agreement to sell or otherwise transfer to a third party the PRV within one year of its receipt, the shareholders' agent may appoint a financial advisor to conduct a process to sell the PRV. If the Company determines in its sole discretion to use the PRV, the Company shall give the shareholders' agent written notice thereof and shall pay to the Scioderm stockholders, option holders, and warrant holders \$100 million. The inability to sell the PRV after complying with the provisions, shall not give rise to any payment.

The fair value of the contingent consideration payments on the acquisition date was \$259.0 million. This was an estimate based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a range of discount rates between 0.4% and 1.1% as interpolated from the U.S. Treasury constant maturity yield curve over the time frame for clinical and regulatory milestones and a range of discount rates between 1.0% and 2.2% for revenue-based milestones. The range of outcomes and assumptions used to develop these estimates have been updated to better reflect the probability of certain milestone outcomes and updated timelines related to clinical development and

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anticipated approval assumptions as of September 30, 2016 without limitation, the \$5 million milestone paid in the second quarter and milestone payments projected for 2017 (See Note 9. Assets and Liabilities Measured at Fair Value , for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). In April 2016, while the total clinical and regulatory approval milestone payments remain unchanged at \$361 million, the allocation between the clinical and regulatory approval milestone payments were revised as follows: clinical milestones of up to \$81 million and regulatory approval milestones of up to \$280 million. The commercial milestone payments of up to \$257 million remained unchanged. The Company determined the fair value of the contingent consideration to be \$262.2 million at September 30, 2016, of which \$56.0 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of \$9.4 million for the nine months ended September 30, 2016. The expense is recorded in the Consolidated Statement of Operations as the change within fair value of contingent consideration payable.

See Note 9. Assets and Liabilities Measured at Fair Value , for additional discussion regarding fair value measurements of the contingent acquisition consideration payable.

For additional information, see Note 6. Goodwill and Intangible Assets.

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The purchase price allocation was subject to completion of our analysis of the fair value of the assets and liabilities as of the effective date of the acquisition. The final valuation was completed as of December 31, 2015. A substantial portion of the assets acquired consisted of intangible assets related to SD-101. The Company determined that the estimated acquisition-date fair value of the indefinite lived IPR&D related to the SD-101 was \$463.7 million.

**Acquisition of Callidus Biopharma, Inc.**

In November 2013, the Company acquired Callidus a privately-held biologics company focused on developing best-in-class ERTs for LSDs with its lead ERT ATB200 for Pompe disease in late preclinical development. The acquisition of the Callidus assets and technology complements the Company's CHART platform for the development of next-generation ERTs.

The fair value of the contingent acquisition consideration payments on the acquisition date was \$10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. As of September 30, 2016, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes; see Note 9. Assets and Liabilities Measured at Fair Value, for additional discussion regarding fair value measurements of the contingent acquisition consideration payable. The Company determined the fair value of the contingent consideration to be \$10.0 million at September 30, 2016, of which \$9.6 million relates to ATB-200 Pompe program. The change in fair value of contingent consideration payable is recorded in the Consolidated Statement of Operations. All of the contingent consideration is payable beyond the next twelve months. During the second quarter of 2016, the Company reached the first clinical milestone, which was the dosing of the first patient in a Phase 1 or 2 study. The milestone for this event was \$6.0 million which was paid in Company stock during the second quarter of 2016, resulting in \$6.1 million impact on stockholder's equity.

For further information, see Note 6. Goodwill and Intangible Assets.

**Note 6. Goodwill and Intangible Assets**

In connection with the acquisitions discussed in Note 5. Acquisitions, the Company has recognized goodwill of \$197.8 million. The following table represents the changes in goodwill for the nine months ended September 30, 2016:

	( in millions)	
Balance at December 31, 2015	\$	197.8
Change in goodwill		
Balance at September 30, 2016	\$	197.8

In connection with the acquisitions discussed in Note 5. Acquisitions, the Company recognized IPR&D of \$486.7 million. Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a

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reduction in the fair value of the IPR&D assets below their respective carrying amounts. The following table represents the changes in IPR&D for the nine months ended September 30, 2016:

	( in millions)	
Balance at December 31, 2015	\$	486.7
Change in IPR&D		
Balance at September 30, 2016	\$	486.7

Goodwill and intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. For the nine months ended September 30, 2016, there were no indicators of impairment.

### Note 7. Debt Instruments and Related Party Transactions

In October 2015, the Company entered into a Note and Warrant Purchase Agreement (the October 2015 Purchase Agreement ) with Redmile Capital Fund, LP and certain of its affiliates, whereby it sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its unsecured promissory notes ( Notes ) and (b) five-year warrants ( Warrants ) for approximately 1.3 million shares of Common Stock. The payment terms under the purchase agreement contains two installments, the first \$15.0 million in October 2017 and the balance \$35.0 million in October 2020. Interest was payable at 4.1% on a monthly

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basis over the term of the loan. The promissory notes are recorded as due to related party on the consolidated balance sheets. Due to the embedded redemption (put and/or call) features in the note agreement, it was determined that the fair value of the warrants should be bifurcated from the value of the notes payable and recorded as a debt discount. The relative fair value of the warrants and the debt discount as related to the October 2015 purchase agreement was determined to be \$8.8 million.

On February 19, 2016, the Company entered into a Note and Warrant Purchase Agreement (the February 2016 Purchase Agreement) with Redmile for an aggregate amount of up to \$75.0 million. The Company has agreed with Redmile that in full consideration of the purchase price for the notes issued under the February 2016 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purchase Agreement and the Company paid Redmile the interest accrued thereunder. Upon entering the February 2016 Agreement Redmile beneficially owned approximately 10% of the Company's outstanding shares of Common Stock and warrants. As such the promissory notes are presented as due to related party on the consolidated balance sheets.

Pursuant to the February 2016 agreement, at closing, it sold, on a private placement basis (a) \$50.0 million aggregate principal amount of unsecured promissory notes (Initial Notes) and (b) five year warrants to purchase up to 37 shares of the Company's Common Stock for every \$1,000 of the principal amount of Initial Notes purchased (Initial Warrants), for an aggregate of up to 1,850,000 shares of Common Stock issuable under the Initial Warrants. The payment terms contain two installments, the first \$15.0 million in October 2017 and the balance \$35.0 million in October 2021. The interest rate is 3.875% and payable upon of maturity. This transaction was accounted for as a debt modification in accordance with ASC 470-50. The incremental fair value between the warrants that were cancelled and the February issued warrants of \$3.5 million was recorded as additional unamortized debt discount on the balance sheet and added to the prior warrant balance within equity. The debt discount will be amortized over the life of the Initial Notes using the effective interest rate method.

On June 30, 2016, following the positive CHMP opinion for migalastat in Europe and the subsequent EC marketing approval, the Company entered into the Amended Purchase Agreement with Redmile, which joined GCM to the February 2016 Purchase Agreement. There was no change to the previously issued debt. Pursuant to the Amended Purchase Agreement, the Company sold an additional \$30.0 million unsecured promissory notes and five year warrants to purchase up to 42 shares of the Company's Common Stock for every \$1,000 of the principal amount of additional Notes purchased (Additional Warrants), for an aggregate of up to 1,260,000 shares of Common Stock. The \$30.0 million payment is due in October 2021. The interest rate is 3.875% and payable upon of maturity.

The fair value of the warrants was determined to be \$3.8 million and recorded as a debt discount. The fair value of the warrants were calculated utilizing the Black-Scholes valuation model using the following six inputs: (1) the closing price of the Company's Common Stock on the day of evaluation of \$5.46; (2) the exercise price of the warrants of \$7.06; (3) the remaining term of the warrants of 5 years; (4) the volatility of the Company's Common Stock for the five year term of 86.02%; (5) the annual rate of dividends of 0%; and (6) the risk-free rate of return of 1.01%.

The outstanding debt as of September 30, 2016 between Redmile and GCM as of September 30, 2016 is as follows (in thousands):

Creditor	Gross amount of debt	Net unamortized discount	Net carrying value of debt
RedMile	\$ 55,000	\$ (10,953)	\$ 44,047
GCM	25,000	(3,023)	21,977
<b>Total Debt</b>	<b>\$ 80,000</b>	<b>\$ (13,976)</b>	<b>\$ 66,024</b>

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The debt discount amortization for the three and nine months ended September 30, 2016 was \$0.7 million and \$1.7 million, respectively.

As of September 30, 2016, the total warrants were recorded at \$16.1 million. See Note 8. Stockholders' Equity for more details.



Table of Contents**Note 8. Stockholders Equity***Common Stock and Warrants*

As of September 30, 2016, the Company was authorized to issue 250 million shares of Common Stock. Dividends on Common Stock will be paid when, and if, declared by the board of directors. Each stockholder is entitled to vote on all matters that are appropriate for stockholder voting and is entitled to one vote for each share held.

Beginning in April 2016 and through July 2016, the Company sold 15.0 million shares of Common Stock under an at-the-market ( ATM ) equity program with Cowen and Company, LLC ( Cowen ) acting as sales agent. Cowen was compensated at a fixed commission rate up to 3.0%. The ATM sales agreement resulted in net proceeds of \$97.1 million, after Cowen s commission of \$2.7 million and other expenses of \$0.2 million. The Company has completed all sales under the ATM equity program.

As discussed in Note 7. Debt instruments and Related Party Transactions, the Company issued approximately 1.8 million and 1.3 million of warrants in February 2016 and June 2016, respectively.

The total outstanding warrants as of September 30, 2016 is as follows (in thousands):

<b>Creditor</b>	<b>Warrant shares</b>	<b>Warrant fair value</b>
RedMile	2,060	\$ 12,927
GCM	1,050	3,149
<b>Total warrants</b>	<b>3,110</b>	<b>\$ 16,076</b>

The closing balance of the warrants was \$16.1 million as of September 30, 2016 on the Consolidated Balance Sheets.

*Nonqualified Cash Plan*

The Company s Deferral Plan, (the Deferral Plan ) provides certain key employees and members of the Board of Directors as selected by the Compensation Committee, with an opportunity to defer the receipt of such participant s base salary, bonus and director s fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Internal Revenue Code of 1986, as amended.

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Deferred compensation amounts under the Deferral Plan as of September 30, 2016 were approximately \$1.3 million, as compared to \$0.7 million on December 31, 2015 and are included in other long-term liabilities. Deferral Plan assets as of September 30, 2016 were \$1.3 million, as compared to \$0.7 million as of December 31, 2015 and are classified as trading securities. The Deferred Plan assets are recorded at fair value with changes in the investments' fair value recognized in the period they occur. The income from investment for the three and nine months ended September 30, 2016 and 2015 was de minimis. Unrealized gain approximated \$30 thousand and \$37 thousand for the three and nine months ended September 30, 2016, respectively as compared to unrealized loss of \$54 thousand and \$64 thousand for the three and nine months ended September 30, 2015, respectively.

Table of Contents**Equity Incentive Plan***Stock Option Grants*

The fair value of the stock options granted is estimated on the date of grant using a Black-Scholes option pricing model with the following weighted-average assumptions:

	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Expected stock price volatility	81.6%	74.4%	81.3%	75.4%
Risk free interest rate	1.2%	1.7%	1.5%	1.7%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

A summary of the Company's stock options for the nine months ended September 30, 2016 is as follows:

	Number of Shares ( in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value ( in millions)
Balance at December 31, 2015	11,729.2	\$ 7.11		
Options granted	4,829.6	\$ 7.70		
Options exercised	(371.7)	\$ 4.05		
Options forfeited	(413.9)	\$ 8.79		
Balance at September 30, 2016	15,773.2	\$ 7.32	7.5 years	\$ 24.9
Vested and unvested expected to vest September 30, 2016	14,718.8	\$ 7.24	7.4 years	\$ 24.1
Exercisable at September 30, 2016	7,395.4	\$ 6.44	5.9 years	\$ 16.3

As of September 30, 2016, the total unrecognized compensation cost related to non-vested stock options granted was \$35.8 million and is expected to be recognized over a weighted average period of 2.9 years.

*Restricted Stock Units*

A summary of non-vested Restricted Stock Units ( RSU ) activity under the Company's Amended and Restated 2007 Equity Incentive Plan for the nine months ended September 30, 2016 is as follows:

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	Number of Shares ( in thousands)	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value ( in millions)
Non-vested units as of December 31, 2015	478.5	\$ 10.38		
Granted	582.7	\$ 6.21		
Vested	(199.3)	\$ 7.25		
Forfeited	(23.2)	\$ 8.52		
Non-vested units as of September 30, 2016	838.7	\$ 8.28	2.69	\$
Non-vested units expected to vest at September 30, 2016	838.7	\$ 8.28	2.69	\$

For the nine months ended September 30, 2016, 199,266 of the RSUs vested and all non-vested units are expected to vest over their normal term.

As of September 30, 2016, there was \$4.8 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 2.69 year.

Table of Contents*Compensation Expense Related to Equity Awards*

The following table summarizes information related to compensation expense recognized in the statements of operations related to the equity awards (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Equity compensation expense recognized in:				
Research and development expense	\$ 2,109	\$ 1,232	\$ 6,011	\$ 3,224
General and administrative expense	2,229	1,505	7,076	3,705
Total equity compensation expense	\$ 4,338	\$ 2,737	\$ 13,087	\$ 6,929

**Note 9. Assets and Liabilities Measured at Fair Value**

The Company's financial assets and liabilities are measured at fair value and classified within the fair value hierarchy, which is defined as follows:

*Level 1* Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

*Level 2* Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

*Level 3* Inputs that are unobservable for the asset or liability.

A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of September 30, 2016, are identified in the following table (in thousands):

	Level 1	Level 2	Total
<b>Assets:</b>			
Cash/ money market funds	\$ 33,115	\$	\$ 33,115
Corporate debt securities		79,618	79,618
Commercial paper		98,653	98,653

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Certificate of deposit			1,013		1,013
Market exchanged mutual funds			1,325		1,325
	\$	33,115	\$	180,609	\$ 213,724

	Level 2	Level 3	Total
<b>Liabilities:</b>			
Contingent consideration payable		\$ 272,190	\$ 272,190
Derivative liability	\$ 324		324
Deferred compensation plan liability	1,350	\$	1,350
	\$ 1,674	\$ 272,190	\$ 273,864

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A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2015, are identified in the following table (in thousands):

	Level 1	Level 2	Total
<b>Assets:</b>			
Cash/ money market funds	\$ 69,485	\$	\$ 69,485
Corporate debt securities		118,474	118,474
Commercial paper		25,724	25,724
Certificate of deposit		350	350
Market exchanged mutual funds		658	658
	\$ 69,485	\$ 145,206	\$ 214,691

	Level 2	Level 3	Total
<b>Liabilities:</b>			
Contingent consideration payable		274,077	274,077
Deferred compensation plan liability	667		667
	\$ 667	\$ 274,077	\$ 274,744

### ***Cash, Money Market Funds and Marketable Securities***

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available-for-sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the nine months ended September 30, 2016. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the nine months ended September 30, 2016.

### ***Note Payable to Related Party and GCM***

In connection with the notes payable to Redmile, as disclosed in Note 7. Debt Instruments and Related Party Transactions, and Warrants as disclosed in Note 8. Stockholders' Equity, the Company recorded the notes as a liability of \$66.0 million on an amortized cost basis.

The warrants issued in connection with the Amended Purchase Agreement were determined to be a component of equity based on the current accounting guidance. As such, these warrants which are considered Level 3 instruments were valued at the issuance date using the Black-Scholes valuation model using the following six inputs: (1) the closing price of the Company's Common Stock on the day of evaluation of \$5.46; (2) the exercise price of the warrants of \$7.06; (3) the remaining term of the warrants of 5 years; (4) the volatility of the Company's Common Stock for the five year term of 86.02%; (5) the annual rate of dividends of 0%; and (6) the risk-free rate of return of 1.01%. The Black-Scholes value of the warrants was \$3.8 million.

As of September 30, 2016, the warrants are recorded at \$16.1 million and the notes at \$66.0 million, net of discount of \$14.0 million.

*Contingent Consideration Payable*

The contingent consideration payable resulted from the acquisitions of Scioderm and Callidus, as discussed in Note 5. Acquisitions. The most recent valuation was determined using a probability weighted discounted cash flow valuation approach. Using this approach, expected future cash flows are calculated over the expected life of the agreement, are discounted, and then exercise scenario probabilities are applied. The valuation is performed quarterly. Gains and losses are included in the statement of operations.

As discussed in Note 5. Acquisitions, on July 5, 2016, the Company entered into the MiaMed Agreement with MiaMed. MiaMed is a pre-clinical biotechnology company focused on developing protein replacement therapy ( product candidate ) for CDKL5 and related diseases. Under the terms of the MiaMed Agreement, the Company agreed to pay up to an additional \$83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of \$89.5 million. The MiaMed Agreement was accounted for as an asset acquisition and as such the Company determined that a liability for future milestone payments is not required to be recorded until the actual contingencies are met and will be recorded to research and development expenses when the contingency is resolved.



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The contingent consideration payable for Scioderm and Callidus has been classified as a Level 3 recurring liability as its valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approach the estimated fair value could be significantly higher or lower than the fair value the Company determined. The Company may be required to record losses in future periods, including expenses related to CDKL5.

The following significant unobservable inputs were used in the valuation of the contingent consideration payable to former Scioderm stockholders:

Contingent Consideration Liability	Fair value as of September 30, 2016	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	\$238.3 million	Probability weighted discounted cash flow	Discount rate	0.5%-3.1%
			Probability of achievement of milestones	66.5% -100.0%
			Projected year of payments	2017-2019
			Revenue volatility	58%
Revenue-based milestones	\$23.9 million	Monte Carlo	Probability of achievement of milestones	66.5%
			Discount rate	0.9%-1.7%
			Projected year of payments	2019-2029

The following significant unobservable inputs were used in the valuation of the contingent consideration payable to former Callidus shareholders for the ATB-200 Pompe program:

Contingent Consideration Liability	Fair value as of September 30, 2016	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	\$9.6 million	Probability weighted discounted cash flow	Discount rate	10.5%
			Probability of achievement of milestones	30%-43%
			Projected year of payments	2018-2022

Contingent consideration liabilities are remeasured to fair value each reporting period using projected revenues, discount rates, probabilities of payment and projected payment dates. Projected contingent payment amounts related to clinical and regulatory based milestones are discounted

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back to the current period using a discounted cash flow model. Revenue-based payments are valued using a monte-carlo valuation model, which simulates future revenues during the earn-out-period using management's best estimates. Projected revenues are based on our most recent internal operational budgets and long-range strategic plans. Increases in projected revenues and probabilities of payment may result in higher fair value measurements. Increases in discount rates and the time to payment may result in lower fair value measurements. Increases or decreases in any of those inputs together, or in isolation, may result in a significantly lower or higher fair value measurement. There is no assurance that any of the conditions for the milestone payments will be met.

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The following table shows the change in the balance of contingent consideration payable for the nine months ended September 30, 2016 and 2015, respectively (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Balance, beginning of the period	\$ 276,300	\$ 11,800	\$ 274,077	\$ 10,700
Additions, from business acquisitions		269,884		269,884
Payment of contingent consideration in cash			(5,000)	
Payment of contingent consideration in stock			(6,115)	
Change in fair value change during the period, included in Statement of Operations	(4,110)	1,300	9,228	2,400
Balance, end of the period	\$ 272,190	\$ 282,984	\$ 272,190	\$ 282,984

*Deferred Compensation Plan- Investment and Liability*

The Company considers its investments in marketable securities, as available-for-sale and classifies these assets and related liability within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities.

*Foreign Currency Exchange Rate Exposure*

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016, the Company entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company did not designate this forward contract as a hedging instrument under applicable accounting guidance and, therefore, the change in fair value is recorded in the Consolidated Statements of Operations. The forward contract settles in monthly installments with the final installment settlement in June 2017.

There were no outstanding forward contracts at December 31, 2015.

For the three and nine months ended September 30, 2016, the Company recognized a gain of \$22 thousand and a loss of \$324 thousand, respectively, related to the derivative instruments not designated as hedging instruments in the Consolidated Statements of Operations and the corresponding liability of \$324 thousand is recorded as other current liability in the Consolidated Balance Sheet.

The impact of gains and losses on foreign exchange contracts not designated as hedging instruments related to changes in the fair value of assets and liabilities denominated in foreign currencies are generally offset by net foreign exchange gains and losses, which are also included on the Consolidated Statements of Operations in other income (expense), net for all periods presented. When the Company enters into foreign exchange

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contracts not designated as hedging instruments to mitigate the impact of exchange rate volatility in the translation of foreign earnings, gains and losses will generally be offset by fluctuations in the U.S. Dollar translated amounts of each Income Statement account in current and/or future periods.

Table of Contents**Note 10. Restructuring Charges**

In December 2013, the Company initiated and completed a facilities consolidation effort, closing one of its leased locations in San Diego, CA. The Company recorded a charge of \$0.7 million related to the net present value of the net future minimum lease payments at the cease-use date.

The following table summarizes the restructuring charges and utilization for the nine months ended September 30, 2016 (in thousands):

	Balance as of December 31, 2015	Charges	Cash Payments	Fair Value Adjustments	Balance as of September 30, 2016
Facilities consolidation	\$ 118	\$	\$ (187)	\$ 69	\$

As of September 30, 2016, the lease had expired and the Company completed the restructuring process.

**Note 11. Basic and Diluted Net Loss per Common Share**

The Company calculates net loss per share as a measurement of the Company's performance while giving effect to all dilutive potential common shares that were outstanding during the reporting period. The Company has a net loss for all periods presented; accordingly, the inclusion of Common Stock options and warrants would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted earnings per share are the same.

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss per common share:

(In thousands, except per share amounts)	Three months Ended September 30,		Nine months Ended September 30,	
	2016	2015	2016	2015
<b>Historical</b>				
Numerator:				
Net loss	\$ (46,654)	\$ (37,800)	\$ (141,395)	\$ (89,222)
Denominator:				
Weighted average common shares outstanding basic and diluted	140,656,109	118,724,882	131,675,690	104,885,956

Dilutive common stock equivalents would include the dilutive effect of common stock options, restricted stock units and warrants for common stock equivalents. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect. The table below presents potential shares of common stock that were excluded from the computation as they

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were anti-dilutive using the treasury stock method (in thousands):

	As of September 30,	
	2016	2015
Options to purchase common stock	15,773	11,574
Outstanding warrants, convertible to common stock	3,110	
Unvested restricted stock units	839	748
Total number of potentially issuable shares	19,722	12,322

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**Note 12. Commitments and Contingencies**

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company's Common Stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. On May 26, 2016, the Court consolidated these lawsuits into a single action and appointed a lead plaintiff. The lead plaintiff filed a Consolidated Amended Complaint on July 11, 2016. Defendants' motion to dismiss was fully briefed on October 28, 2016.

The Company believes that it has meritorious defenses and intends to defend the lawsuits vigorously. These lawsuits and any other related lawsuits are subject to inherent uncertainties, and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain, the Company could be forced to expend significant resources in the defense of these lawsuits and it may not prevail.

On or about November 2, 2015, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against the individuals who serve on the Amicus Board of Directors. Amicus itself was named as a nominal defendant. The derivative lawsuit alleged claims for breach of state law fiduciary duties, waste of corporate assets, and unjust enrichment based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. On February 19, 2016, the complaint was dismissed by the Court and plaintiffs have not refiled.

On or about March 3, 2016, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against various officers and directors of the Company. Amicus itself is named as a nominal defendant. The derivative lawsuit alleges similar facts and circumstances as the three purported securities class action lawsuits described above and further alleges claims for breach of state law fiduciary duties, waste of corporate assets, unjust enrichment, abuse of control, and gross mismanagement based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. The plaintiff seeks, among other things, to require the Amicus Board to take certain actions to reform its corporate governance procedures, including greater shareholder input and a provision to permit shareholders to nominate candidates for election to the Board, along with restitution, costs of suit and attorney's fees. The parties have entered into a stipulation to stay the time to respond to the derivative complaint until the resolution of any motion to dismiss in the above-referenced securities action.

This lawsuit and any other related lawsuits are subject to inherent uncertainties and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain and the Company could be forced to expend significant resources in the defense of this suit, and the Company may not prevail. The Company is not currently able to estimate the possible cost to it from this matter, as this lawsuit is currently at an early stage and the Company cannot ascertain how long it may take to resolve this matter.

**Note 13. Subsequent Events**

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The Company evaluated events that occurred subsequent to September 30, 2016 and there were no material recognized or non-recognized subsequent events during this period.



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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

**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.

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.

**Program Status**

We have completed two global Phase 3 registration studies of our lead product candidate, migalastat HCl, an orally administered small molecule pharmacological chaperone for the treatment of Fabry disease, an LSD. On May 31, 2016, we announced that we had received full European Commission approval for migalastat HCl, under the product 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 label includes 269 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. In the U.S., discussions with the U.S. Food and Drug Administration ( FDA ) continue and we expect to provide a U.S. regulatory update in the fourth quarter of 2016. For patients with non-amenable mutations, we are leveraging our CHART technology and advanced biologics capabilities to move forward with a proprietary Fabry ERT for co-formulation with migalastat. Master cell banking has been completed and process development work has commenced.

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. In May 2016, the we announced that the European Commission ( EC ) had granted full approval for the oral small molecule pharmacological chaperone Galafold (migalastat) 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 (alpha-galactosidase A deficiency) and who have an amenable mutation. The approved label includes 269 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. We commenced commercial shipments of Galafold in the EU in the second quarter of 2016 and recognized net product sales of \$2.1 million in the third quarter of 2016.

We are also in Phase 3 clinical development of a novel topical medicine, SD-101, for the treatment of the genetic connective tissue disorder EB, for which no other pharmacological therapies are currently approved. We have also initiated a clinical study in patients with Pompe disease, another LSD, to investigate our novel treatment paradigm that consists of ATB200, a uniquely engineered recombinant human acid alpha-glucosidase ( rhGAA ) enzyme with an optimized carbohydrate structure to enhance uptake, co-administered with a pharmacological chaperone, AT2221, to improve activity and stability. Leveraging our biologics capabilities and platform technologies, we are also investigating

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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 advanced product pipeline uniquely position us at the forefront of developing therapies to potentially address significant unmet needs for devastating rare and orphan diseases.

### **Migalastat for Fabry Disease**

#### *Overview*

Our most advanced technology, migalastat, is a small molecule pharmacological chaperone for the treatment of Fabry disease that has been approved for use in the European Union ( EU ) under the brand name Galafold<sup>®</sup> for patients with Fabry disease with an amenable mutation. Outside of the EU, migalastat is an investigational product. As an orally administered monotherapy, migalastat is designed to bind to and stabilize an endogenous alpha-galactosidase A ( alpha-Gal A ) enzyme in those patients with genetic mutations identified as amenable in a GLP cell-based amenability assay. We are also developing the use of migalastat in combination with a novel Fabry ERT for patients who have non-amenable genetic mutations.

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Patients with the fatal, x-linked Fabry disease have an inherited deficiency of the alpha-Gal A enzyme that would normally degrade the lipid substrate globotriaosylceramide in the lysosome. Genetic mutations that cause changes in the amino acid sequence of alpha-Gal A result in an unstable enzyme that does not efficiently fold into its correct three-dimensional shape and cannot be trafficked properly in the cell, even if it has the potential for biological activity. Migalastat is an oral small molecule pharmacological chaperone that is designed to bind to and stabilize a patient's own endogenous target protein. This is considered a precision medicine because migalastat targets only patients with amenable mutations.

We have completed two Phase 3 global registration studies of migalastat monotherapy. We have reported Phase 3 data in both treatment-naïve patients ( Study 011 ) and ERT-switch patients ( Study 012 ). Results from these studies have shown that treatment with migalastat results in reductions in disease substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations in a validated GLP amenability assay.

*IV Migalastat Co-Formulated with ERT for Fabry Disease*

For patients with non-amenable mutations, we are leveraging our CHART technology and advanced biologics capabilities to move forward with 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 also 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. 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 up to 150 patients with 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.

We also held a series of discussions with the Dermatology Division of the U.S. FDA regarding proposed revisions to the statistical analysis plan while remaining blinded to the Phase 3 ESSENCE study. Based on conversations with FDA and written communication received from the agency, the FDA has agreed to our proposed revisions. Importantly, the FDA agreed that Time to Target Wound Closure may be elevated from a secondary endpoint to a co-primary endpoint (together with the previously specified primary endpoint Proportion of Patients with Target Wound Closure). Based on this feedback, we believe that study success could potentially be based on achievement of one or both co-primary endpoints, assuming appropriate analytical methodology, and that the overall likelihood of study success has been improved.

*SD-101 for EB: Regulatory Pathway*

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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 and are aligned on the design of the current Phase 3 study and the global regulatory pathway forward for SD-101 based on a single Phase 3 registration-directed study. The FDA agreed to a rolling NDA in the U.S., which was initiated in the fourth quarter of 2015. Following the Phase 2b study, our 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 EMA approval process and must be accepted prior to a submission of an MAA in the EU. Results from the Phase 3 study are anticipated in the first half of 2017 to support marketing applications for SD-101 in the U.S., EU and other regions.

### *Novel ERT for Pompe Disease*

We are leveraging our biologics capabilities and CHART platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered rhGAA enzyme (designated ATB200 ) with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone AT2221 to improve activity and stability.

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In the fourth quarter of 2015, we initiated the Phase 1/2 clinical study ATB200-02 to investigate our novel Pompe disease treatment paradigm in Pompe disease patients. The key features of this Phase 1/2 study include:

- Open-label, dose-escalation to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of intravenous ATB200 co-administered with oral AT2221;
  - Subjects in the first cohorts will be adult Pompe disease patients switched from currently marketed ERT;
  - Primary treatment period will be 18 weeks, with all patients eligible to enroll in an open-label extension study; and
- Interim data from this study are anticipated in the fourth quarter of 2016.

Following a positive data safety monitoring board review of the safety data in the initial group of ambulatory ERT-switch patients ( Cohort 1 ), we have been cleared to enroll non-ambulatory ERT-switch and naïve patients ( Cohorts 2-3 ). Data from the first four patients in Cohort 1 are on track by year-end 2016. Additional ATB200-02 study data in Cohorts 2-3, as well as initial extension-study data on ambulatory ERT-switch patients, are anticipated throughout first half of 2017.

***CDKL5***

We are researching a potential first-in-class protein replacement therapy approach for CDKL5 deficiency in preclinical studies. CDKL5 (cyclin-dependent kinase-like 5) 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.

**Acquisitions**

*MiaMed, Inc*

On July 5, 2016, we entered into an Agreement and Plan of Merger (the MiaMed Agreement ) with MiaMed, Inc., ( MiaMed ) and certain other parties signatory thereto. MiaMed is a pre-clinical biotechnology company focused on developing protein replacement therapy for CDKL5 and

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related diseases. Under the terms of the MiaMed Agreement, in connection with the closing of the transactions contemplated by the MiaMed Agreement, the former holders of MiaMed's capital stock received an aggregate of \$6.5 million, comprised of (i) approximately \$1.8 million in cash (plus MiaMed's cash and cash equivalents at closing and less any of MiaMed's unpaid third-party fees and expenses related to the transaction), and (ii) 825,603 shares of the Company's common stock. In addition, we also agreed to pay up to an additional \$83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of \$89.5 million.

### *Scioderm, Inc.*

In September 2015, we acquired Scioderm, Inc., ( Scioderm ), which strengthens our pipeline significantly with the addition of a novel, late-stage, proprietary topical medicine and potential first-to-market therapy for EB (SD-101). This investigational product was granted FDA breakthrough therapy designation in 2013, based on results from Phase 2 studies for the treatment of lesions in patients suffering with EB. SD-101 is currently being investigated in a Phase 3 study to support global regulatory submissions and was the first-ever treatment in EB clinical studies to show improvements in wound closure across all major EB subtypes.

We acquired Scioderm in a cash and stock transaction. At closing, the Company paid Scioderm stockholders, option holders and warrant holders approximately \$223.9 million, of which approximately \$141.1 million was paid in cash and approximately \$82.8 million was paid through the issuance of 5.9 million newly issued Amicus shares. We agreed to pay up to an additional \$361 million to Scioderm stockholders, option holders and warrant holders upon achievement of certain clinical and regulatory milestones and \$257 million upon achievement of certain sales milestones. If SD-101 is approved, EB qualifies as a rare pediatric disease and we will request a Priority Review Voucher. If the Priority Review Voucher is obtained and subsequently sold, we will pay Scioderm stockholders, option holders and warrant holders the lesser of \$100 million in the aggregate or 50% of the proceeds of such sale.

During the second quarter of 2016, we reached the first event-based milestone, which was the 50% enrollment of patients. The milestone payment for this event was \$5.0 million which was paid in cash during the second quarter of 2016.

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*Callidus Biopharma, Inc.*

In November 2013, we entered into a merger agreement with Callidus, a privately held biotechnology company. Callidus was engaged in developing a next-generation Pompe ERT and complementary enzyme targeting technologies.

In connection with our acquisition of Callidus, we agreed to issue an aggregate of 7.2 million shares of our common stock to the former stockholders of Callidus. In addition, we will be obligated to make additional payments to the former stockholders of Callidus upon the achievement of certain clinical milestones of up to \$35 million and regulatory approval milestones of up to \$105 million set forth in the merger agreement, provided that the aggregate merger consideration shall not exceed \$130 million. We may, at our election, satisfy certain milestone payments identified in the merger agreement aggregating \$40 million in shares of our common stock. The milestone payments not permitted to be satisfied in common stock (as well as any payments that we are permitted to, but chooses not to, satisfy in common stock), as a result of the terms of the merger agreement, will be paid in cash.

During the second quarter of 2016, we reached the first clinical milestone, which was the dosing of the first patient in a Phase 1 or 2 study. The milestone payment for this event was \$6.0 million, which was paid in common stock of the Company, during the second quarter of 2016.

**Critical Accounting Policies, Significant Judgments and Estimates and Business Combinations**

The discussion and analysis of our financial condition and results of operations are based on our financial statements, which we have prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

There were no significant changes during the quarter ended September 30, 2016 to the items that we disclosed as our significant accounting policies and estimates described in Note 2. Summary of Significant Accounting Policies to the Company's financial statements as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2015. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations. Additionally, the Company added new policies on inventory and product sales in the current quarter.

*Revenue Recognition*

We recognize revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when there is persuasive evidence of an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collection of the amounts due are reasonably assured and the Company has no further performance obligations.

*Net Product Sales*

Our net product sales consist solely of sales of Galafold for the treatment of Fabry disease in the EU. We have recorded revenue on sales where Galafold is available either on a commercial basis or through a reimbursed early access program. Orders for Galafold are generally received from pharmacies and the ultimate payor is typically a government authority.

We record revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

*Inventories*

Until regulatory approval of Galafold, we expensed all manufacturing costs as research and development expense. Upon regulatory approval, we began capitalizing costs related to the purchase and manufacture of Galafold.

Inventories are stated at the lower of cost or market determined by the first-in, first-out method. We periodically review inventories to identify slow-moving or obsolete inventory based on projected sales activity as well as product shelf-life. In evaluating the recoverability of inventories produced, we consider the probability that revenue will be obtained from the future sale of the related inventory and inventory value is written down for inventory quantities in excess of the expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statements of operations.



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We analyze our inventory levels to identify inventory that may expire prior to sale, inventory that has a cost basis in excess of its estimated realizable value, or inventory in excess of expected sales requirements. Although the manufacturing of our products is subject to strict quality controls, certain batches or units of product may no longer meet quality specifications or may expire, which would require adjustments to our inventory values. Inventory becomes obsolete when it has aged past its shelf-life, cannot be recertified and is no longer usable or able to be sold, or the inventory has been damaged. In such instances, a full reserve is taken against such inventory.

In the future, reduced demand, quality issues or excess supply beyond those anticipated by management may result in an adjustment to inventory levels, which would be recorded as an increase to cost of product sales. The determination of whether or not inventory costs will be realizable requires estimates by our management. A critical input in this determination is future expected inventory requirements based on our internal sales forecasts which we compare to inventory on hand after consideration of expiration dates.

*Research and Development Expenses*

We expect to continue to incur substantial research and development expenses as we continue to develop our product candidates and explore new uses for our pharmacological chaperone technology. Research and development expense consists of:

- internal costs associated with our research and clinical development activities;
- payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants;
- technology license costs;
- manufacturing development costs;
- personnel-related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and

- facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies.

We have multiple research and development projects ongoing at any one time. We utilize our internal resources, employees and infrastructure across multiple projects. We record and maintain information regarding external, out-of-pocket research and development expenses on a project-specific basis.

We expense research and development costs as incurred, including payments made to date under our license agreements. We believe that significant investment in product development is a competitive necessity and plan to continue these investments in order to realize the potential of our product candidates.

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The following table summarizes our principal product development programs, including the related stages of development for each product candidate in development, and the out-of-pocket, third party expenses incurred with respect to each product candidate (in thousands):

Projects	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Third party direct project expenses				
<b>Monotherapy Studies</b>				
Migalastat (Fabry Disease Phase 3)	\$ 2,726	\$ 3,798	\$ 9,503	\$ 11,736
SD-101 (EB-Epidermolysis Bullosa Phase 3)	3,503		6,979	
<b>Combination Studies</b>				
ATB200 + AT2221 (Pompe Disease Phase 2)	5,946	7,523	12,535	15,503
Fabry CHART (Fabry Disease Preclinical)	91	461	282	1,587
Neurodegenerative Diseases (Preclinical)	6,490	3	6,490	6
Total third party direct project expenses	\$ 18,756	\$ 11,785	\$ 35,789	\$ 28,832
<b>Other project costs (1)</b>				
Personnel costs	9,782	6,598	27,349	18,133
Other costs (2)	3,919	2,588	11,025	7,353
Total other project costs	\$ 13,701	\$ 9,186	\$ 38,374	\$ 25,486
Total research and development costs	\$ 32,457	\$ 20,971	\$ 74,163	\$ 54,318

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- (1) Other project costs are leveraged across multiple projects.
  - (2) Other costs include facility, supply, overhead, and licensing costs that support multiple projects.

### *Stock Option Grants*

In accordance with the applicable guidance, we measure stock-based compensation at a fair value which is determined by measuring the cost of employee services received in exchange for an award of equity instruments based upon the grant date fair value of the award. We chose the straight-line attribution method for allocating compensation costs and recognized the fair value of each stock option on a straight-line basis over the vesting period of the related awards.

We use the Black-Scholes option pricing model when estimating the value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was based on our historical volatility since our initial public offering in May 2007. The expected life was determined using the simplified method as described in ASC Topic 718, Accounting for Stock Compensation, which is the midpoint between the vesting date and the end of the contractual term. The risk-free interest rate was based on the U.S. Treasury yield in effect at the date of grant. Forfeitures are estimated based on expected turnover as well as a historical analysis of actual option forfeitures.

The weighted average assumptions used in the Black-Scholes option pricing model are as follows:

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	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Expected stock price volatility	81.6%	74.4%	81.3%	75.4%
Risk free interest rate	1.2%	1.7%	1.5%	1.7%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

*Restricted Stock Units*

Beginning in 2014, the Compensation Committee made awards of restricted stock units ( RSUs ) to certain of our employees. The RSUs are generally subject to graded vesting and are contingent on an employee's continued service on such date. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. We expense the cost of the RSUs, which is determined to be the fair market value of the shares of Common Stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

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*Warrants*

On February 19, 2016, we entered into a Note and Warrant Purchase Agreement (the "February 2016 Purchase Agreement") with Redmile Capital fund, LP and certain funds and accounts managed or advised by it (collectively referred to as "Redmile") whereby we sold, on a private placement basis, (a) \$50 million aggregate principal amount of unsecured promissory notes and (b) five-year warrants to purchase up to 37 shares of our Common Stock for every \$1,000 of the principal amount of notes purchased by each purchaser, for an aggregate of up to 1,850,000 shares of Common Stock issuable under the warrants. We agreed with Redmile that in full consideration of the purchase price for the notes issued under the October 2015 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purchase Agreement and we paid Redmile any unpaid interest accrued thereunder. Upon entering the February 2016 Agreement Redmile beneficially owned approximately 10% of the Company's outstanding shares of Common Stock and warrants. As such the promissory notes are presented as due to related party on the consolidated balance sheets.

On June 30, 2016, following the positive CHMP opinion for migalastat in Europe and the subsequent EC marketing approval, we entered into a Joinder to and Amendment of Note and Warrant Purchase Agreement (the "Amended Purchase Agreement") with Redmile. Such amendment joined GCM Grosvenor Special Opportunities Master Fund, Ltd ("GCM") to the February 2016 Purchase Agreement. There were no changes to the previously issued debt. Pursuant to the Amended Purchase Agreement, we sold an additional \$30 million unsecured promissory notes and five year warrants to purchase up to 42 shares of the our Common Stock for every \$1,000 of the principal amount of additional Notes purchased, for an aggregate of up to 1,260,000 shares of Common Stock issuable under the additional warrants. The payment is due in October 2021. The interest rate is 3.875% and payable upon of maturity.

*Nonqualified Cash Deferral Plan*

Our Cash Deferral Plan (the "Deferral Plan") provides certain key employees and other service providers as selected by the Compensation Committee, with an opportunity to defer the receipt of such participant's base salary, bonus and director's fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Internal Revenue Code of 1986, as amended.

The amounts deferred under the Deferral Plan are included in the non-current assets within the accompanying consolidated balance sheet. All of the investments held in the Deferral Plan are classified as trading securities and recorded at fair value with changes in the investments' fair value recognized in the period they occur. The corresponding liability for the Deferral Plan is included in other non-current liability in our consolidated balance sheets.

*Foreign Currency Transactions and Derivative Financial Instruments*

We transact business in various foreign countries and therefore we are subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016, we entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. We did not designate this forward contract as a hedging instrument under applicable accounting guidance and, therefore, the change in fair value is recorded in the other income/(expense) line in the Consolidated Statements of Operations, with the corresponding liability in other current liability on the

Consolidated Balance Sheet.

The forward contract will settle in June 2017.

There were no outstanding forward contracts at December 31, 2015.

For the three and nine months ended September 30, 2016, we recognized a gain of \$22 thousand and a loss of \$324 thousand, respectively, related to the foreign currency forward contract not designated as hedging instruments.

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**Results of Operations**

*Three months Ended September 30, 2016 Compared to Three months Ended September 30, 2015*

*Net product sales.* Net product sales were \$2.1 million for the three months ended September 30, 2016 due to marketing approval of Galafold granted in May 2016.

*Cost of goods sold.* Cost of goods sold includes manufacturing costs as well as estimated royalties associated with sales of our product. Cost of goods sold as a percentage of net sales was 16.2% for the three months ended September 30, 2016.

*Research and Development Expense.* Research and development expense was \$32.5 million during the three months ended September 30, 2016, representing an increase of \$11.5 million or 54.8% from \$21.0 million for the three months ended September 30, 2015. The increase in research and development costs was primarily due to increases in clinical research costs of \$9.4 million due to the advancement and enrollment of clinical studies, and \$3.5 million related to the EB program. Also included in the research and development expenses was \$6.5 million which represents a one-time expense associated with the acquisition of the CDKL5 asset. Other increases were in external program support of \$4.0 million, of which \$3.1 million related to personnel costs.

*Selling, General and Administrative Expense.* Selling, general and administrative expense was \$17.5 million for the three months ended September 30, 2016, representing an increase of \$2.1 million or 13.6% from \$15.4 million for the three months ended September 30, 2015. The increase was primarily due to personnel costs of \$3.7 million in support of the commercial organization for the launch of Galafold, partially offset by a decrease in professional fees of \$3.0 million.

*Changes in Fair Value of Contingent Consideration Payable.* For the three months ended September 30, 2016, we recorded gain of \$4.1 million representing a change of \$5.4 million from the \$1.3 million of expense for the three months ended September 30, 2015. The change in the fair value resulted primarily from a decrease in the Callidus contingent consideration of \$1.8 million, and a decrease in the Scioderm contingent consideration of \$3.6 million. The change in the fair value was impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods, discount rates and changes in the allocation of the contingent milestones.

*Restructuring Charges.* Restructuring charges arose from the corporate restructuring implemented in the fourth quarter of 2013. This measure was intended to reduce costs and to align our resources with our key strategic priorities. The increase to the restructuring expense was \$11 thousand for three months ended September 30, 2016, as compared to

\$7 thousand for the three months ended September 30, 2015, and was due to the change in fair value of the future minimum lease payments. As of September 30, 2016, all of the restructuring activities have been completed.

*Depreciation Expense.* Depreciation expense was \$0.9 million for the three months ended September 30, 2016, representing an increase of \$0.5 million as compared to \$0.4 million for the three months ended September 30, 2015. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2016.

*Interest Income.* Interest income was \$0.5 million for the three months ended September 30, 2016, representing an increase of \$0.2 million from \$0.3 million for the three months ended September 30, 2015. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

*Interest Expense.* Interest expense was approximately \$1.5 million for three months ended September 30, 2016, representing an increase of \$1.5 million from \$17 thousand for the three months ended September 30, 2015. Interest expense was higher due to the \$50 million notes payable borrowed in October 2015 and the related revised agreement in February 2016, as well as the \$30 million unsecured notes borrowed in June 2016, partially offset by the early retirement of a \$15 million secured loan in June 2015.

*Other Expense.* Other expenses for the three months ended September 30, 2016 was \$0.9 million, as compared to other expenses of \$0.1 million for the three months ended September 30, 2015. The change was primarily from unrealized losses on foreign exchange transactions.

*Tax benefit:* For the three months ended September 30, 2016, the Company recorded a discrete income tax benefit of \$0.3 million related to the reduction in its valuation allowances to reflect the income tax associated with the gain on foreign currency translation recorded in the Consolidated Statements of Comprehensive loss. A corresponding income tax benefit was also recorded in the Consolidated Statements of Operations.



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*Nine Months Ended September 30, 2016 Compared to Nine Months Ended September 30, 2015*

*Net product sales.* Net product sales were \$2.1 million for the nine months ended September 30, 2016 due to marketing approval of Galafold granted in May 2016.

*Cost of goods sold.* Cost of goods sold includes manufacturing costs as well as estimated royalties associated with sales of our product. Cost of goods sold as a percentage of net sales was 16.2% for the nine months ended September 30, 2016.

*Research and Development Expense.* Research and development expense was \$74.2 million during the nine months ended September 30, 2016, representing a change of \$19.9 million or 36.6% from \$54.3 million for the nine months ended September 30, 2015. The increase in research and development costs was primarily due to increases in clinical research costs of \$10.4 million, due to the advancement and enrollment of clinical studies, primarily the EB program for \$7.0 million. Also included in the research and development expenses was \$6.5 million which represents a one-time expense associated with the acquisition of the CDKL5 asset. Other increases were in personnel costs of \$9.2 million.

*Selling, general and Administrative Expense.* Selling, general and administrative expense was \$52.5 million for the nine months ended September 30, 2016, representing an increase of \$22.4 million or 74.4% from \$30.1 million for the nine months ended September 30, 2015 in support of the commercial organization for the launch of Galafold. The increase was due to personnel costs of \$12.8 million and professional fees of \$5.4 million.

*Changes in Fair Value of Contingent Consideration Payable.* For the nine months ended September 30, 2016, we recorded expense of \$9.2 million representing an increase of \$6.8 million from the \$2.4 million of expense for the nine months ended September 30, 2015. The change in the fair value resulted primarily from an increase in the Scioderm contingent consideration of \$9.4 million, partially offset by decrease in Callidus contingent consideration of \$2.6 million. The change in the fair value is impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods, discount and changes in the allocation of the contingent milestones. In the second quarter of 2016, we made milestone payments of \$11.1 million.

*Loss from Extinguishment of Debt.* In the nine months ended September 30, 2016, we did not recognize a loss from extinguishment of debt, as compared to \$1.0 million in the nine months ended September 30, 2015 arising from the early extinguishment of a \$15 million secured loan.

*Restructuring Charges.* Restructuring charges arose from the corporate restructuring implemented in the fourth quarter of 2013. This measure was intended to reduce costs and to align our resources with our key strategic priorities. The increase to the restructuring expense was \$69 thousand for nine months ended September 30, 2016 as compared to \$44 thousand for the nine months ended September 30, 2015, and was due to the change in fair value of the future minimum lease payments. As of September 30, 2016, all the restructuring activities have been completed.

*Depreciation Expense.* Depreciation expense was \$2.3 million for the nine months ended September 30, 2016, representing an increase of \$1.0 million as compared to \$1.3 million for the nine months ended September 30, 2015. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2016.

*Interest Income.* Interest income was \$1.1 million for the nine months ended September 30, 2016, representing an increase of \$0.5 million from \$0.6 million for the nine months ended September 30, 2015. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

*Interest Expense.* Interest expense was approximately \$3.5 million for nine months ended September 30, 2016, representing an increase of \$2.8 million from \$0.7 million for the nine months ended September 30, 2015. Interest expense was higher due to the \$50 million notes payable borrowed in October 2015 and the related revised agreement in February 2016, as well as the \$30 million notes borrowed in June 2016, partially offset by the early retirement of a \$15 million secured loan in June 2015.

*Other Expense.* Other expenses for the nine months ended September 30, 2016 was \$3.2 million, as compared to \$0.1 million for the nine months ended September 30, 2015. The change was primarily from losses on foreign exchange transactions.

*Tax benefit:* For the nine months ended September 30, 2016, the Company recorded a discrete income tax benefit of \$0.7 million related to the reduction in its valuation allowances to reflect the income tax associated with the gain on foreign currency translation recorded in the Consolidated Statements of Comprehensive loss. A corresponding income tax benefit was also recorded in the Consolidated Statements of Operations.

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**Liquidity and Capital Resources**

*Source of Liquidity*

Beginning in April 2016 and through July 2016, we sold 15.0 million shares of Common Stock under an at-the-market ( ATM ) equity program with Cowen and Company, LLC ( Cowen ) acting as sales agent. Cowen was compensated at a fixed commission rate up to 3.0%. The ATM sales agreement resulted in net proceeds of \$97.1 million, after Cowen s commission of \$2.7 million and other expenses of \$0.2 million. We have completed all sales under the ATM equity program.

On June 30, 2016, following the positive CHMP opinion for migalastat in Europe and the subsequent EC marketing approval, we entered into the Amended Purchase Agreement with Redmile. Such amendment joined GCM to the February 2016 Purchase Agreement. Pursuant to the Amended Purchase Agreement, we sold an additional \$30 million unsecured promissory notes and five year warrants to purchase up to purchase up to 42 shares of our Common Stock, par value \$0.01 per share for every \$1,000 of the principal amount of additional notes purchased, for an aggregate of up to 1,260,000 shares of Common Stock issuable from the additional warrants. The payment is due in October 2021. The interest rate is 3.875% and payable upon of maturity.

As a result of our significant research and development expenditures as well as expenditures to build a commercial organization to support the launch of Galafold, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$693.1 million of gross proceeds from our stock offerings, \$130.0 million from investments by collaborators and non-refundable license fees from those collaborations.

As of September 30, 2016, we had cash and cash equivalents and marketable securities of \$212.4 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

*Net Cash Used in Operating Activities*

Net cash used in operations for the nine months ended September 30, 2016 was \$118.5 million, due primarily to the net loss for the nine months ended September 30, 2016 of \$141.4 million and the change in operating assets and liabilities of \$10.1 million. The change in operating assets and liabilities was primarily due to decrease in accounts payable and accrued expenses of \$2.9 million, partially offset by increases in inventory of \$3.5 million, prepaid assets of \$2.8 million and other non-current assets of \$0.7 million.

Net cash used in operations for the nine months ended September 30, 2015 was \$64.6 million, due primarily to the net loss for the nine months ended September 30, 2015 of \$89.2 million and non-cash items such as stock based compensation of \$6.9 million, the change in fair value of the contingent consideration of \$2.4 million and the loss on the extinguishment of debt of \$1.0 million. In addition there was change in operating

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assets and liabilities of \$12.9 million. The change in operating assets and liabilities was due to an increase in accrued expenses of \$14.1 million, in prepaid assets of \$0.7 million and in other non-current assets of \$0.5 million.

### *Net Cash Used in Investing Activities*

Net cash used in investing activities for the nine months ended September 30, 2016 was \$39.9 million and reflects \$199.8 million for the purchase of marketable securities, \$5.5 million for the acquisition of property and equipment, partially offset by \$165.5 million for the sale and redemption of marketable securities.

Net cash used in investing activities for the nine months ended September 30, 2015 was \$230.8 million. Net cash used in investing activities reflects \$220.9 million for the purchase of marketable securities, \$141.1 million paid to the former Scioderm shareholders as part of the Scioderm acquisition, \$2.2 million for the acquisition of property and equipment, partially offset by \$133.4 million for the sale and redemption of marketable securities.

### *Net Cash Provided by Financing Activities*

Net cash provided by financing activities for the nine months ended September 30, 2016 was \$122.4 million. Net cash provided by financing activities reflects \$97.1 million from issuance of Common Stock under the ATM program, \$30.0 million as proceeds from the Amended Purchase agreement and \$1.5 million from exercise of stock options, partially offset by \$5.0 million paid to Scioderm as contingent consideration and \$1.0 million from vesting of RSUs.

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Net cash provided by financing activities for the nine months ended September 30, 2015 was \$290.7 million. Net cash provided by financing activities reflects \$243.0 million from issuance of common stock, \$50.0 million from proceeds from debt with Redmile Group, \$10.7 million from exercise of stock options and \$4.0 million from exercise of warrants, partially offset by \$15.3 million from paying the secured loan and \$1.7 million from vesting of RSU.

***Funding Requirements***

We expect to incur losses from operations for the foreseeable future primarily due to research and development expenses, including expenses related to conducting clinical trials. Our future capital requirements will depend on a number of factors, including:

- the progress and results of our clinical trials of our drug candidates, including migalastat HCl;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new 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 lysosomal storage diseases;
- the future results of ongoing or later clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and market acceptance of SD-101;
- 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;

- 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 or technologies;
- our ability to successfully incorporate Scioderm and its products and technology into our business, including the possibility that the expected benefits of the transaction will not be fully realized by us or may take longer to realize than expected; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

While we generated revenue from product sales in the third quarter of 2016, in the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We may seek additional funding through public or private financings of debt or equity. We believe that our existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into the fourth quarter of 2017.

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***Financial Uncertainties Related to Potential Future Payments***

*Milestone Payments / Royalties*

We acquired exclusive worldwide patent rights to develop and commercialize migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with MSSM. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2018 in the U.S. and 2019 in Europe and Japan for monotherapy. If we develop a product for combination therapy of specific pharmacological chaperone such as migalastat plus an ERT for certain Lysosomal Storage Disorders such as Fabry disease and a patent issues from the pending MSSM applications covering such a combination therapy(ies) expiration for the combination product(s) will be 2024. Under this agreement, to date we have paid no upfront or annual license fees and have no milestone or future payments other than royalties on net sales.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For migalastat, we will owe royalties only to MSSM and will owe no milestone payments.

In November 2013, we entered into the Revised Agreement with GlaxoSmithKline ( GSK ), pursuant to which we have obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between us and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from us to GSK. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones up to \$40 million, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. In addition, because we reacquired worldwide rights to migalastat, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement. We will owe royalties to MSSM in addition to those owed to GSK.

As part of the merger agreement with Scioderm, we have agreed to pay up to an additional \$361 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain clinical and regulatory milestones, and \$257 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain sales milestones. If SD-101 is approved, EB qualifies as a rare pediatric disease and we will request a Priority Review Voucher. If the Priority Review Voucher is obtained and subsequently sold, we will pay Scioderm stockholders, option holders and warrant holders the lesser of \$100 million in the aggregate or 50% of the proceeds of such sale. In April 2016, while the total clinical and regulatory approval milestone payments remain unchanged at \$361 million, the allocation between the clinical and regulatory approval milestone payments were revised as follows: clinical milestones of up to \$81 million and regulatory approval milestones of up to \$280 million. The commercial milestone payments of up to \$257 million remained unchanged. During the second quarter of 2016, we reached the first event based milestone for Scioderm, which was the 50% enrollment of patients in the phase 3 study. The milestone payment for this event was \$5.0 million which was paid in cash during the second quarter of 2016.

As part of the acquisition of Callidus, we will be obligated to make additional payments to the former stockholders of Callidus upon the achievement by the Company of certain clinical milestones of up to \$35 million and regulatory approval milestones of up to \$105 million as set forth in the merger agreement, provided that the aggregate consideration shall not exceed \$130 million. We may, at our election, satisfy certain milestone payments identified in the merger agreement aggregating \$40 million in shares of our Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Select Market for the ten trading days immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the we are permitted to, but choose not to, satisfy in Common Stock), as a result of the terms of the merger agreement, the

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rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash. During the second quarter of 2016, we reached the first clinical milestone for Callidus, which was the dosing of the first patient in a Phase 1 or 2 study. The milestone payment for this event was \$6.0 million which was paid in the Company's stock during the second quarter of 2016.

As part of the acquisition of MiaMed, we will be obligated to make additional payments to the former stockholders of MiaMed upon the achievement by the Company of certain clinical milestones of up to \$8 million, regulatory approval milestones of up to \$10 million, and commercial milestones up to \$65 million. Any milestone payment may be satisfied in cash, shares of Common Stock, or a combination of both. The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the we are permitted to, but choose not to, satisfy in Common Stock), as a result of the terms of the merger agreement, the rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash. No milestone payments in connection with the acquisition of MiaMed have been paid.

To date, we have not made any royalty payments on sales of our products, however, we expect that royalty payments to MSSM and GSK will be made starting in the fourth quarter of 2016.



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***Recent Accounting Pronouncements***

Please refer to Note 2. Summary of Significant Accounting Policies, in our Notes to Consolidated Financial Statements.

**ITEM 3. Quantitative and Qualitative Disclosures about Market Risk**

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At September 30, 2016, we held \$212.4 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. At September 30, 2016, our cash, cash equivalents and available for sale securities were all due on demand or within one year. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

We have operated primarily in the U.S. with international operations increasing since the last quarter of 2015. We do conduct some clinical activities with vendors outside the U.S. While most expenses are paid in U.S. dollars, we now have increased transactions of expenses and cash flows in foreign currencies that are exposed to changes in foreign currency rates. Foreign currency forward contracts used to offset these exposures are not designated as hedges.

**ITEM 4. CONTROLS AND PROCEDURES**

As of the end of the period covered by this Quarterly Report on Form 10-Q, an evaluation of the effectiveness of our disclosure controls and procedures (pursuant to Rule 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act )) was carried out under the supervision of our Principal Executive Officer and Principal Financial Officer, with the participation of our management. Based on that evaluation, the Principal Executive Officer and the Principal Financial Officer concluded that, as of the end of such period, our disclosure controls and procedures are effective in recording, processing, summarizing and reporting, on a timely basis, information required to be disclosed by us in the reports that we file or submit under the Exchange Act and are effective in ensuring that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

During the fiscal quarter covered by this report, there has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the last fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for the District of New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company's Common Stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. On May 26, 2016, the Court consolidated these lawsuits into a single action and appointed a lead plaintiff. The lead plaintiff filed a Consolidated Amended Complaint on July 11, 2016. Defendants' motion to dismiss was fully briefed on October 28, 2016.

We believe that we have meritorious defenses and intend to defend the lawsuits vigorously. These lawsuits and any other related lawsuits are subject to inherent uncertainties, and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain, we could be forced to expend significant resources in the defense of these lawsuits and we may not prevail.

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On or about November 2, 2015, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against the individuals who serve on the Amicus Board of Directors. Amicus itself was named as a nominal defendant. The derivative lawsuit alleged claims for breach of state law fiduciary duties, waste of corporate assets, and unjust enrichment based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. On February 19, 2016, the complaint was dismissed by the Court and plaintiffs have not refiled.

On or about March 3, 2016, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against various officers and directors of the Company. Amicus itself is named as a nominal defendant. The derivative lawsuit alleges similar facts and circumstances as the three purported securities class action lawsuits described above and further alleges claims for breach of state law fiduciary duties, waste of corporate assets, unjust enrichment, abuse of control, and gross mismanagement based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. The plaintiff seeks, among other things, to require the Amicus Board to take certain actions to reform its corporate governance procedures, including greater shareholder input and a provision to permit shareholders to nominate candidates for election to the Board, along with restitution, costs of suit and attorney's fees. The parties have entered into a stipulation to stay the time to respond to the derivative complaint until the resolution of any motion to dismiss in the above-referenced securities action.

These lawsuits and any other related lawsuits are subject to inherent uncertainties, and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain, we could be forced to expend significant resources in the defense of these lawsuits and we may not prevail.

**ITEM 1A. RISK FACTORS**

The following risk factors and other information included in this Quarterly Report on Form 10-Q, including our financial statements, the notes thereto and the section entitled Management's Discussion and Analysis of Financial Condition and Results of Operations, should be carefully considered. If any of the following risks actually occurs, our business, financial condition, operating results, prospects and ability to accomplish our strategic objectives could be materially harmed. As a result, the trading price of our common stock could decline and you could lose all or part of your investment. Investing in our common stock involves a high degree of risk. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we currently deem less significant may also impair our business operations and the market price of our common stock.

**Risks Related to Our Financial Position and Need for Additional Capital**

*We have incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.*

We are an early commercial-stage pharmaceutical company. To date, we have focused on developing our first product, migalastat HCl. Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable. Since our inception we have not generated any material revenue from product sales. Although

the European Commission has granted full approval for the oral small molecule pharmacological chaperone migalastat HCl 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 (alpha-galactosidase A deficiency) and who have an amenable mutation, neither the United States Food and Drug Administration, or FDA nor any other non-EU foreign regulatory authority has granted regulatory approval to any of our product candidates, and we continue to incur significant research, development, commercialization and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. For the year ended December 31, 2015, we reported a net loss of \$132.1 million, and we had an accumulated deficit of \$579.6 million at December 31, 2015.

We expect to continue to incur losses for the foreseeable future, and we expect these losses to increase as we:

- continue our development and commercialization of, and seek regulatory approvals for, our product and product candidates in the United States, the European Union, and other foreign countries, as applicable;
- conduct additional clinical trials and/or further analysis of pre-existing clinical data to support the New Drug Application, or NDA, of migalastat HCl in the United States if required by the FDA;
- continue communicating with the EMA, as necessary, regarding post-marketing requirements for migalastat HCl;
- initiate the regulatory submission process for marketing approval of migalastat HCl outside of the United States and EU;

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- build our commercial infrastructure so that it is capable of supporting product sales, marketing and distribution of migalastat HCl in the EU, US and other territories in which we may receive regulatory approval;
- continue our ongoing Phase 3 clinical trial of SD-101 for the treatment of epidermolysis bullosa, or EB; and
- continue our preclinical studies and clinical trials on the use of pharmacological chaperones co-formulated or co-administered with enzyme replacement therapy, or ERT, for Fabry, Pompe, and other lysosomal storage disorders, or LSDs.

We may encounter unforeseen expenses, difficulties, complications, delays, and other unknown factors that may adversely affect our business. The size of our future losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. If any of our product candidates fails in clinical trials or does not gain regulatory approval, or if approved, fails to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

*We currently generate no revenue from the sale of products and may never become profitable.*

We began the commercial launch of our first product, migalastat HCl, in May 2016. Accordingly, we have not generated any material revenue from product sales. Our ability to generate material revenue and become profitable depends upon our ability to successfully commercialize our existing product and product candidates, or product candidates that we may in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for our product candidates, we do not know when any of these product candidates will generate revenue for us, if at all. Our ability to generate revenue from our current or future product and product candidates depends on a number of factors, including our ability to:

- successfully complete development activities and obtain regulatory approval for, and successfully commercialize, migalastat HCl;
- develop a commercial organization capable of sales, marketing, and distribution for migalastat HCl and any product candidates we intend to market, if we receive regulatory approval, in the countries where we have chosen to commercialize the product candidates ourselves;
- manufacture commercial quantities of our products at acceptable cost levels;

- obtaining a commercially viable price for our products;
- obtain coverage and adequate reimbursement from third-parties, including government payors;
- successfully satisfy post-marketing requirements that the FDA, EMA, or other foreign regulatory authorities may impose if migalastat HCl or any of our other product candidates receive regulatory approval;
- successfully complete development activities, including the necessary preclinical studies and clinical trials, with respect to product candidates other than migalastat HCl;
- complete and submit NDAs to the FDA and obtain regulatory approval for our product candidates including migalastat HCl; and
- complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities.

In addition, because of the numerous risks and uncertainties associated with product development, including that our product candidates may not advance through development or achieve the safety and efficacy endpoints of applicable clinical trials, we are unable to predict the timing or amount of increased expenses, or when or if we will be able to achieve or maintain profitability. Furthermore, we anticipate incurring significant costs associated with commercializing these products.

Even if we are able to generate revenues from the sale of our products, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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*If we require substantial additional capital to fund our operations and we fail to obtain necessary financing, we may be unable to complete the commercialization of our product and development and commercialization of our product candidates.*

Our operations have consumed substantial amounts of cash. We expect to continue to spend substantial amounts to advance the clinical development of our product candidates, and launch and commercialize our product and product candidates for which we may receive regulatory approval, including building our own commercial organization. We believe that our existing cash and cash equivalents will be sufficient to fund our operations into 2017, including the commercialization of migalastat HCl in the EU, and the continuation of our development of our other product candidates. However, we may require substantial additional capital for the commercialization of our product and further development and commercialization of our product candidates.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital in sufficient amounts, when required or on acceptable terms, we could also be required to:

- significantly delay, scale back, or discontinue the development or the commercialization of our product or product candidates or one or more of our other research and development initiatives;
- seek collaborators for migalastat HCl or one or more of our current or future product candidates at an earlier stage than otherwise would be desirable, or on terms that are less favorable than might otherwise be available;
- relinquish or license on unfavorable terms our rights to our technologies, product or product candidates that we otherwise would seek to develop or commercialize ourselves; or
- significantly curtail operations.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed elsewhere in this Risk Factors section. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the costs of commercialization activities, including establishing sales, marketing, and distribution capabilities for migalastat HCl and any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own;

- the scope, progress, results, and costs of preclinical development, laboratory testing, and clinical trials for our product candidates and any other product candidates that we may in-license or acquire;
- the cost of manufacturing drug supply for our preclinical studies and clinical trials, including the significant cost of new Fabry ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the outcome, timing, and cost of the regulatory approval process by the FDA, EMA, and other foreign regulatory authorities, including the potential for regulatory authorities to require that we perform more studies than those that we currently anticipate for our product and product candidates;
- the cost of filing, prosecuting, defending, and enforcing any patent claims and other intellectual property rights;
- the cost and timing of completion of existing or expanded commercial-scale outsourced manufacturing activities;
- the cost of defending any claims asserted against us, including the pending securities class action lawsuit brought against us in the United States District Court for the District of New Jersey and shareholder derivative lawsuits against us in the Superior Court of New Jersey Middlesex County;
- the emergence of competing technologies and other adverse market developments;
- the extent to which we acquire or invest in additional businesses, products, and technologies; and
- the cost to integrate our recent acquisitions of Scioderm, Inc., or Scioderm, and MiaMed, Inc., or MiaMed, their products and technologies into our business.



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***Raising additional capital may cause dilution to our existing stockholders, restrict our operations, or require us to relinquish rights to our technologies, migalastat HCl or product candidates.***

We may seek additional capital through a combination of private and public equity offerings, debt financings, receivables or royalty financings, strategic collaborations and alliances, and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of existing stockholders. Debt, receivables, and royalty financings may be coupled with an equity component, such as warrants to purchase stock, which could also result in dilution of our existing stockholders' ownership. For example, stockholders may experience dilution if the holders of the warrants issued in connection with our private placement in October 2015 and February 2016 exercise their warrants. The incurrence of additional indebtedness beyond our existing indebtedness with Redmile Capital Fund, LP, or Redmile, could also result in increased fixed payment obligations and could also result in certain restrictive covenants, such as limitations on our ability to incur further debt, limitations on our ability to acquire or license intellectual property rights, and other operating restrictions that could have a material adverse effect on our ability to conduct our business and may result in liens being placed on our assets and intellectual property. If we were to default on any of our indebtedness, we could lose such assets and intellectual property. If we raise additional funds through strategic collaborations and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to migalastat HCl or our product candidates, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market our technologies that we would otherwise prefer to develop and market ourselves.

On October 1, 2015, we entered into a Note and Warrant Purchase Agreement with Redmile and certain of its affiliates whereby we sold, on a private placement basis, (i) \$50.0 million aggregate principal amount of unsecured promissory notes, and (ii) 1,349,998 warrants that have a term of five years. The payment terms under the purchase agreement consist of two installments, the first \$15.0 million is due in October 2017 and the \$35.0 million balance is due in October 2020. Interest is payable at 4.1% on a monthly basis over the term of the loan.

On February 19, 2016, we entered into another Note and Warrant Purchase Agreement with Redmile Group, LLC and certain funds and accounts managed or advised by it, whereby we sold, on a private placement basis, (i) \$50,000,000 aggregate principal amount of unsecured promissory notes and (b) five-year warrants to purchase up to 37 shares of our common stock for every \$1,000 of the principal amount of notes purchased by each purchaser, for an aggregate of up to 1,850,000 shares of common stock issuable under the warrants. The notes bear interest at 3.875%. Of the \$50,000,000 of notes, \$15,000,000 of the aggregate principal notes will be issued by us and will mature on October 1, 2017, which is the same maturity as the original October 1, 2015 note. \$35,000,000 of the aggregate principal notes will be issued by Amicus Therapeutics UK Limited and mature on October 1, 2021, a one-year increase in maturity from the original October 1, 2015 note. For each tranche, interest will accrue but go unpaid until final maturity. We agreed with Redmile that in full consideration of the purchase price for the notes issued under the October 2015 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purchase Agreement and we will pay Redmile any unpaid interest accrued thereunder.

There can be no assurance that our cash and cash equivalents, together with funds generated by our operations and any future financings, will be sufficient to satisfy our debt payment obligations. Our inability to generate funds or obtain financing sufficient to satisfy our debt payment obligations may result in such obligations being accelerated by our lenders, which would likely have a material adverse effect on our business, financial condition and results of operations.

***We have a limited operating history, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

We commenced operations in February 2002. Our operations to date have been limited to organizing and staffing our company, acquiring and developing our technology and undertaking preclinical studies and clinical trials of our most advanced product candidates, including our first product, migalastat HCl. We have not yet generated any material commercial sales for any of our product candidates. We have not yet demonstrated our ability to obtain global regulatory approvals (including the US), manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history. In addition, because we were successful in obtaining from the European Union full approval for migalastat HCl, we will need to transition from a company with a research focus to a company capable of supporting commercial activities. We have not demonstrated an ability to commercialize a product and may not be successful in such a transition.

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*We may not realize all of the anticipated benefits of the acquisitions of Scioderm or MiaMed.*

The success of our acquisitions of Scioderm and MiaMed will depend, in part, on our ability to realize the anticipated growth opportunities and synergies from combining the businesses of our company, Scioderm and MiaMed. Our ability to realize these benefits, and the timing of this realization, depend upon a number of factors and future events, many of which we, Scioderm and MiaMed, individually or collectively, cannot control. These factors and events include:

- integrating Scioderm's and MiaMed's technology platforms into our company;
- reliance on the representations and warranties given by the former Scioderm management and board and former MiaMed management and board which may prove to be incomplete, inaccurate or misleading.
- reliance on the opinions of neutral or other third parties referred to us by the former Scioderm management and board and the former MiaMed management and board prior to the acquisitions that may prove to be incomplete, inaccurate or misleading.
- obtaining and maintaining intellectual property rights relating to the Scioderm and MiaMed technologies;
- enforcing our intellectual property rights covering SD-101 against third party manufacturers or compounding pharmacies;
- third party manufacturers or compounding pharmacies designing around our intellectual property covering SD-101;
- effectively consolidating research and development operations;
- retaining and attracting key employees;
- consolidating corporate and administrative functions;

- any delays in enrollment in on-going clinical trials for SD-101;
- the success of on-going or later clinical trials for SD-101;
- maintaining new chemical entity exclusivity and/or orphan drug market exclusivity; and
- minimizing the diversion of management's attention from ongoing business concerns.

Acquisitions involve risks, including inaccurate assessment of undisclosed, contingent, or other liabilities or problems. Following the completion of the mergers, the surviving corporations, which are now wholly owned subsidiaries of our company, possess not only all of the assets, but also all of the liabilities of Scioderm and MiaMed. It is possible that undisclosed, contingent, or other liabilities or problems may arise in the future of which we were previously unaware. These undisclosed liabilities could have an adverse effect on our business, financial condition, and prospects.

***We may acquire other assets or businesses, or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, increase our debt, or cause us to incur significant expense.***

As part of our business strategy, we may continue to pursue acquisitions of assets or businesses, or strategic alliances and collaborations, to expand our existing technologies and operations. We may not identify or complete these transactions in a timely manner, on a cost-effective basis, or at all, and we may not realize the anticipated benefits of any such transaction, any of which could have a detrimental effect on our financial condition, results of operations, and cash flows. We may not be able to find suitable acquisition candidates, and if we make any acquisitions, we may not be able to integrate these acquisitions successfully into our existing business and we may incur additional debt or assume unknown or contingent liabilities in connection therewith. Integration of an acquired company or assets may also disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, especially the acquisition of commercial assets, and require management resources that would otherwise focus on developing our existing business. We may not be able to find suitable collaboration partners or identify other investment opportunities, and we may experience losses related to any such investments.

To finance any acquisitions or collaborations, we may choose to issue debt or shares of our common stock as consideration. Any such issuance of shares would dilute the ownership of our stockholders. If the price of our common stock is low or volatile, we may not be able to acquire other assets or companies or fund a transaction using our stock as consideration. Alternatively, it may be necessary for us to raise additional funds for acquisitions through public or private financings. Additional funds may not be available on terms that are favorable to us, or at all.

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*Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.*

As of December 31, 2015, we had federal and state net operating loss carry forwards, or NOLs, of approximately \$406.1 million and \$382.7 million, respectively. The federal carry forward will expire in 2028 through 2035. Most of the state carry forwards generated prior to 2009 expired in 2015. The remaining state carry forwards including those generated from 2009 through 2015 will expire in 2029 through 2035 due to a change in the New Jersey state law regarding the net operating loss carry forward period. Under Section 382 of the Internal Revenue Code of 1986, as amended, or Section 382, if a corporation undergoes an ownership change, generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOLs and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income may be limited. We may experience ownership changes in the future as a result of shifts in our stock ownership some of which are outside our control. We completed a detailed study of our NOLs and determined that there was not an ownership change in excess of 50%. Ownership changes in future periods may place additional limits on our ability to utilize net operating loss and tax credit carry forwards. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

**Risks Related to our Products and the Regulatory Approval and Clinical Development of our Product Candidates**

*We depend heavily on the commercial success of our first product, migalastat HCl, in the EU. Moreover, if we are unable to obtain approval from the FDA or other foreign regulatory authorities, or if we are unable to commercialize migalastat HCl successfully, or experience significant delays in doing so, our business could be materially harmed.*

We have invested a significant portion of our efforts and financial resources in the development of migalastat HCl for the treatment of Fabry disease. Our ability to generate material product revenues, which may not occur for the foreseeable future, if ever, will depend heavily on the successful development, regulatory approval, and commercialization of migalastat HCl.

Any delay or impediment in our ability to obtain regulatory approval in any region to commercialize, or, if approved, obtain coverage and adequate reimbursement from third-parties, including government payors, for migalastat HCl may cause us to be unable to generate the revenues necessary to continue our research and development pipeline activities, thereby adversely affecting our business and our prospects for future growth.

Further, the success of migalastat HCl will depend on a number of factors, including the following:

- obtain a sufficiently broad label in each territory that would not unduly restrict patient access;
- receipt of marketing approval for migalastat HCl in the United States;

- building an infrastructure capable of supporting product sales, marketing, and distribution of migalastat HCl in the EU and territories where we pursue commercialization directly;
- establishing commercial manufacturing arrangements with third party manufacturers;
- establishing commercial distribution agreements with third party distributors;
- launching commercial sales of migalastat HCl, where approved, whether alone or in collaboration with others;
- acceptance of migalastat HCl, where approved, by patients, the medical community and third party payors;
- the regulatory approval pathway that we pursue for migalastat HCl in the U.S.;
- effectively competing with other therapies;
- a continued acceptable safety profile of migalastat HCl;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity;
- protecting our rights in our intellectual property portfolio; and
- obtaining a commercially viable price for our products

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If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize migalastat HCl, which would materially harm our business.

***If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product or product candidates, and our ability to generate revenue will be materially impaired.***

Our product and product candidates, including migalastat HCl, and the activities associated with their development and commercialization, including their testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, commercialization and reimbursement are subject to comprehensive regulation by the EMA, the FDA, and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain regulatory approval for our product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only obtained regulatory approval to market one product in one jurisdiction.

We have only limited experience in filing and supporting the applications necessary to obtain marketing approvals for product candidates and are and will need to rely on third party contract research organizations, or CROs, to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that migalastat HCl or any of our other product candidates are not effective or only moderately effective, or have undesirable or unintended side effects, toxicities, safety profiles or other characteristics that preclude us from obtaining marketing approval or that prevent or limit commercial use.

Obtaining approval for our product candidates is highly uncertain and we may fail to obtain regulatory approval in any or all jurisdictions. The review processes and the processes of regulatory authorities, including the FDA, are extensive, lengthy, expensive, and uncertain, and such regulatory authorities may delay, limit, or deny approval of migalastat HCl or any of our other product candidates for many reasons, including, but not limited to:

- our failure to demonstrate to the satisfaction of the applicable regulatory authorities that migalastat HCl or any of our other product candidates are safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance or other efficacy or safety parameters required by the applicable regulatory authorities for approval;
- the applicable regulatory authority may disagree with the number, design, size, conduct, or implementation of our clinical trials or conclude that the data fail to meet statistical or clinical significance;

- the applicable regulatory authority may not find the data from preclinical studies and clinical trials sufficient to demonstrate that the product candidate's clinical and other benefits outweigh its safety risks;
- the applicable regulatory authority may disagree with our interpretation of data from preclinical studies or clinical trials, and may reject conclusions from preclinical studies or clinical trials, or determine that primary or secondary endpoints from clinical trials were not met, or reject safety conclusions from such studies or trials;
- the applicable regulatory authority may not accept data generated at one or more of our clinical trial sites;
- the applicable regulatory authority may determine that we did not properly oversee our clinical trials or follow the regulatory authority's advice or recommendations in designing and conducting our clinical trials;
- an advisory committee, if convened by the applicable regulatory authority, may recommend against approval of our application or may recommend that the applicable regulatory authority require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions, or even if an advisory committee, if convened, makes a favorable recommendation, the respective regulatory authority may still not approve the product candidate; and
- the applicable regulatory authority may identify deficiencies in the chemistry, manufacturing, and control sections of our application, our manufacturing processes, facilities, or analytical methods or those of our third party contract manufacturers, and this may lead to significant delays in the approval of our product candidates or to the rejection of our applications altogether.



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The process of obtaining marketing approvals is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical, or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

***If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of the FDA, EMA, or other foreign regulatory authorities, or do not otherwise produce favorable results, we may experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.***

In connection with seeking marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

For example, if the FDA refuses to accept an NDA for accelerated approval or full approval, or accepts the filing, but ultimately decides not to approve the NDA, we may need to complete additional Phase 3 clinical trial(s) and may need to expend significantly more capital to pursue FDA approval of migalastat HCl. If we are required to conduct additional clinical trials or other testing of migalastat HCl or any other product candidate that we develop beyond those tests and trials that we contemplate; if we are unable to successfully complete our clinical trials or other testing; if the results of these trials or tests are not positive or are only modestly positive; or if there are safety concerns, we may:

- choose not to seek regulatory approval in the U.S.;
- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval 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, including boxed warnings;
- be subject to additional post-marketing testing requirements, safety strategies or restrictions, such as a requirement of a risk evaluation and mitigation strategy, or REMS; or
- have the product removed from the market after obtaining regulatory approval.

*If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential regulatory approval or commercialization of our product candidates could be delayed or prevented.*

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive regulatory approval or commercialize our product candidates, including:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- we may be unable to enroll a sufficient number of patients in our trials to ensure adequate statistical power to detect any statistically significant treatment effects;
- our third party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

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- regulators, institutional review boards, or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators, institutional review boards, or independent ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; or
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the trials.

Our product development costs will increase if we experience delays in testing or regulatory approvals. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates, allow our competitors to bring products to market before we do, or impair our ability to successfully commercialize our product candidates, and so may harm our business and results of operations.

*The regulatory pathway for approval of migalastat HCl in the United States is not yet determined, and, depending on the pathway that the FDA requires us to pursue, or if the FDA refuses to accept for filing our NDA with the existing data, our NDA submission could be significantly delayed or unsuccessful or we may decide to no longer seek approval of migalastat HCl in the United States.*

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We had planned, until on or about October 1, 2015 to submit an NDA for accelerated approval (Subpart H) of migalastat HCl with the FDA in the second half of 2015. Under the FDA's accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening disease or condition that provides meaningful therapeutic benefit to patients over available treatments based upon a surrogate or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA has broad discretion over whether to grant approval based on a surrogate endpoint. The regulatory pathway for migalastat HCl is currently uncertain and we continue to confer with the FDA on the appropriate pathway. There can be no assurance of if and when we will receive guidance on the regulatory pathway or whether the FDA will accept an NDA based on existing data under either an accelerated or full approval pathway, or if accepted, whether the NDA will ultimately be approved. If the FDA refuses to accept an NDA for accelerated approval or full approval, or accepts the filing, but ultimately does not approve the NDA, the FDA may require additional Phase 3 clinical trials beyond those already completed for us to continue to seek FDA approval. If required to complete additional trials, we may choose not to complete those trials or pursue U.S. approval, or if we do, we may need to expend significantly more capital with no assurance of the success of any such clinical trial nor of the FDA's ultimate decision regarding approval of migalastat HCl.

If migalastat HCl is approved by the FDA under the accelerated approval regulations, it will be subject to rigorous post-marketing compliance requirements, including the completion of a Phase 4 or post-approval clinical trial(s) to confirm the effect on the clinical endpoint, and FDA review of all promotional materials prior to their dissemination. If we fail to promptly conduct any required post-approval study, do not confirm a clinical benefit during the post-marketing study(ies), other evidence shows that migalastat HCl is not shown to be safe or effective under the conditions of use, or we disseminate promotional materials relating to migalastat HCl that are found by the FDA to be false and misleading, the FDA could seek to withdraw migalastat HCl from the market on an expedited basis.

*If we experience delays or difficulties in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.*

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. Each of our diseases that our lead product candidates are intended to treat are characterized by small patient populations, which could result in slow enrollment of clinical trial participants. For example, the entry criteria for our Phase 3 clinical trial in migalastat HCl for Fabry disease to support approval in the United States (Study 011) required that patients must have a genetic mutation that

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we believe is responsive to migalastat HCl, and may not have received ERT in the past or must have stopped treatment for at least six months prior to enrolling in the study. As a result, enrollment of the clinical trial lasted for over two years. In addition, our competitors have ongoing clinical trials for product candidates that could be competitive with our product candidates. As a result, potential clinical trial sites may elect to dedicate their limited resources to participation in our competitors' clinical trials and not ours, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is affected by other factors including:

- severity of the disease under investigation;
- eligibility criteria for the clinical trial in question;
- perceived risks and benefits of the product candidate under study;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of the company to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients in any of our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

***We may expend our limited resources to pursue a particular product, product candidate or indication and fail to capitalize on a product, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

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Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

We have based our research and development efforts on our Chaperone-Advanced Replacement Therapy ( CHART ) platform technologies to develop next-generation ERT products for Fabry, Pompe, and other LSDs, and on SD-101 for the treatment of EB. Notwithstanding our large investment to date and anticipated future expenditures in proprietary technologies, we have not yet developed, and may never successfully develop, any marketed drugs using this approach. As a result of pursuing the development of our product and product candidates using our proprietary technologies, we may fail to develop products or product candidates or address indications based on other scientific approaches that may offer greater commercial potential or for which there is a greater likelihood of success. Research programs to identify new product candidates require substantial technical, financial and human resources. These research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development.

***Initial results from a clinical trial do not ensure that the trial will be successful and success in early stage clinical trials does not ensure success in later-stage clinical trials.***

We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA or the applicable non-U.S. regulatory authority, in well-designed and conducted clinical trials, that the product candidate is safe and effective and otherwise meets the appropriate standards required for approval for a particular indication. Clinical trials are lengthy, complex and extremely expensive processes with uncertain results. A failure of one or more of our clinical trials may occur at any stage of testing.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. We cannot be assured that these trials will ultimately be successful. In addition, patients may not be compliant with their dosing regimen or trial protocols or they may withdraw from the clinical trial at any time for any reason.

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In addition, while the clinical trials of our product candidates are designed based on the available relevant information, in view of the uncertainties inherent in drug development, such clinical trials may not be designed with focus on indications, patient populations, dosing regimens, safety or efficacy parameters or other variables that will provide the necessary safety or efficacy data to support regulatory approval to commercialize the resulting product candidates. In addition, individual patient responses to the dose administered of a product candidate may vary in a manner that is difficult to predict. Also, the methods we select to assess particular safety or efficacy parameters may not yield statistical precision in estimating our product candidates' effects on study participants. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval.

In addition, our product and certain of our product candidates are based on our active-site pharmacological chaperone technology. To date, we are not aware that any product based on active-site pharmacological chaperone technology has been approved by the FDA. As a result, if the FDA requires different endpoints than the endpoints we anticipate using or have used in our clinical trials, or a different analysis of those endpoints, it may be more difficult for us to obtain, or we may be delayed in obtaining, FDA approval of our product candidates. If we are not successful in commercializing any of our products or product candidates, or are significantly delayed in doing so, our business will be materially harmed.

***We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA and EMA.***

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA and EMA. We have only obtained regulatory approval for one product in one jurisdiction and have only begun the process of commercializing that product. Our limited experience might prevent us from successfully designing or implementing a clinical trial for our product candidates. We have limited experience in conducting and managing the application process necessary to obtain regulatory approvals and we might not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our preclinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our product candidates, or might be significantly delayed in doing so, which will materially harm our business.

***We may not be able to obtain orphan drug exclusivity for our product or product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product or product candidates, or can be classified as a similar medicinal product according to the EMA's regulations, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.***

Regulatory authorities in some jurisdictions, including the EU and the United States, may designate drugs for relatively small patient populations as orphan drugs. We obtained orphan drug designations from the FDA for migalastat HCl for the treatment of Fabry disease in February 2004. We also obtained orphan medicinal product designation in the EU for migalastat HCl in May 2006. SD-101 has also received these designations from the FDA and EMA. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the EMA from approving another marketing application for a similar medicinal product or the FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable market exclusivity period for orphan drugs is ten years in the EU and seven years in the United States. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is no longer justified.

In the EU, a similar medicinal product is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. For a drug such as migalastat HCl, which is composed of small molecules, the FDA defines same drug as a drug that contains the same active moiety and is intended for the same use. Obtaining orphan drug exclusivity for migalastat HCl for these indications, both in the EU and in the United States, may be important to the product candidate s and our CHART program s success. If a competitor obtains orphan drug exclusivity for and approval of a product with the same indication as migalastat HCl before we do and if the competitor s product is the same drug or a similar medicinal product as ours, we could be excluded from the market for a certain period of time.

Even if we obtain orphan drug exclusivity for migalastat HCl for these indications, we may not be able to maintain it. For example, if a competitive product that is the same drug or a similar medicinal product as our product or product candidate is shown to be clinically superior to our product or product candidate, as applicable, any orphan drug exclusivity we have obtained will not block the approval of such competitive product. In addition, orphan drug exclusivity will not prevent the approval of a product that is the same drug as our product or product candidate if the FDA finds that we cannot assure the availability of sufficient quantities of the drug to meet the needs of the persons with the disease or condition for which the drug was designated.



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***Failure to obtain or maintain regulatory approval in international jurisdictions would prevent us from marketing our other products abroad.***

In order to market and sell migalastat HCl and our other products in the EU and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, some countries outside the United States require approval of the sales price of a drug before it can be marketed. In many countries, separate procedures must be followed to obtain reimbursement. We may not obtain marketing, pricing or reimbursement approvals outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. Regulatory approvals in countries outside the United States do not ensure pricing approvals in those countries or in any other countries, and regulatory approvals and pricing approvals do not ensure that reimbursement will be obtained.

***Our product or product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.***

Undesirable side effects caused by our product or product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA, EMA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product or product candidates and generating revenues from their sale. In addition, if we or others identify undesirable side effects caused by our products or product candidates after receipt of marketing approval:

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or additional clinical trials are conducted.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or product candidate or could substantially increase the costs and expenses of commercializing the product or product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

**Risks Related to the Commercialization of our Product Candidates**

*If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product or product candidates, we may not be successful in commercializing migalastat HCl or any product candidate if and when they are approved.*

We are in the process of building our sales and marketing infrastructure and have little experience in the sale and marketing of pharmaceutical products. To achieve commercial success for any approved product, we must continue to develop a sales and marketing organization or outsource these functions to third parties. We plan to establish our own sales and marketing capabilities and to promote migalastat HCl in the EU with a targeted sales force, and to do the same in the United States if and when migalastat HCl or any other product candidate is approved in the United States. There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize migalastat HCl and our product candidates, if and when they are approved by regulatory authorities, including the FDA and EMA, on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;

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- unforeseen costs and expenses associated with creating an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

We may also co-promote our product or product candidates in various markets with pharmaceutical and biotechnology companies in instances where we believe that a larger sales and marketing presence will expand the market or accelerate penetration. If we do enter into arrangements with third parties to perform sales and marketing services, our product revenues will be lower than if we directly sold and marketed our products and any revenues received under such arrangements will depend on the skills and efforts of others.

We may not be successful in entering into distribution arrangements and marketing alliances with third parties. Our failure to enter into these arrangements on favorable terms could delay or impair our ability to commercialize our product and product candidates and could increase our costs of commercialization. Dependence on distribution arrangements and marketing alliances to commercialize our products and product candidates will subject us to a number of risks, including:

- we may not be able to control the amount and timing of resources that our distributors may devote to the commercialization of our product candidates;
- our distributors may experience financial difficulties;
- business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and
- these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

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

Each of the diseases that our product and most advanced product candidates are being developed to address is rare. Our projections of both the number of people who have these diseases, as well as the subset of People with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates.

Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. In addition, as new studies are performed the estimated prevalence of these diseases may change. There can be no assurance that the prevalence of Fabry disease, EB or Pompe disease in the study populations, particularly in these newer studies, accurately reflects the prevalence of these diseases in the broader world population. If our estimates of the prevalence of Fabry disease, EB or Pompe disease, or of the number of patients who may benefit from treatment with our product candidates prove to be incorrect, the market opportunities for our product and product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

*Migalastat HCl or any of our product candidates that receive regulatory approval may fail to achieve the degree of market acceptance by physicians, patients, third party payors and others in the medical community necessary for commercial success.*

Migalastat HCl and any of our other products or product candidates that receive regulatory approval may nonetheless fail to gain sufficient market acceptance by physicians, patients, third party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or any profits from operations. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects;
- the ability to offer our product and product candidates for sale at competitive prices;

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- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third party coverage or reimbursement.

Our ability to negotiate, secure and maintain third party coverage and reimbursement may be affected by political, economic and regulatory developments in the United States, the EU and other jurisdictions. Governments continue to impose cost containment measures, and third party payors are increasingly challenging prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. These and other similar developments could significantly limit the degree of market acceptance of migalastat HCl or any of our product candidates that receive marketing approval.

***We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.***

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product or product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell products for the treatment of lysosomal storage disorders, including Fabry disease. These products include Sanofi Aventis' Fabrazyme® and Shire plc's Replagal®. In addition, Sanofi markets and sells Myozyme® and Lumizyme® for the treatment of Pompe disease. We are also aware of other enzyme replacement and substrate reduction therapies in development by third parties, including Biomarin Pharmaceutical's BMN-701, an ERT in Phase 2/3 development for Pompe disease. Birken AG has completed a Phase 2 trial with Oleogel-S10 for the treatment of EB.

Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or noncompetitive. Our competitors may also obtain FDA, EMA, or other regulatory approval for their products more rapidly than we may obtain approval for ours. We may also face competition from off-label use of other approved therapies. There can be no assurance that developments by others will not render our product candidates or any acquired products obsolete or

noncompetitive either during the research phase or once the products reaches commercialization.

We believe that many competitors, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies, are attempting to develop therapies for many of our target indications. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, prosecuting intellectual property rights and marketing approved products than we do. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs or advantageous to our business. In addition, if we obtain regulatory approvals for our products, manufacturing efficiency and marketing capabilities are likely to be significant competitive factors. We currently have one third party manufacturer and a limited sales force and marketing infrastructure. Further, many of our competitors have substantial resources and expertise in conducting collaborative arrangements, sourcing in-licensing arrangements and acquiring new business lines or businesses that are greater than our own.

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*A variety of risks associated with international operations could materially adversely affect our business.*

Migalastat HCl, and any of our other products or product candidates that are approved for commercialization in the EU, or in other foreign countries, are or will be subject to additional risks related to international operations or entering into international business relationships, including:

- different regulatory requirements for maintaining approval of drugs in foreign countries;
- reduced protection for contractual and intellectual property rights in some countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- noncompliance with the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act 2010 and similar anti-bribery and anti-corruption laws in other jurisdictions;
- tighter restrictions on privacy and the collection and use of patient data; and

- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by both the EU and many of the individual countries in Europe with which we will need to comply. Many U.S.-based biopharmaceutical companies have found the process of marketing their own products in Europe to be very challenging.

***Even if we are able to commercialize migalastat HCl, SD-101 or any other product candidate, the products may become subject to unfavorable pricing regulations, third party coverage and reimbursement practices or healthcare reform initiatives, which would harm our business.***

The regulations and practices that govern marketing approvals, pricing, commercialization, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries, including almost all of the member states of the European Economic Area, require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, including the European market, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact any revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize migalastat HCl or any product candidate successfully also will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the EU and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Prices at which we or our customers seek reimbursement for our products can be subject to challenge, reduction or denial by the government and other payers. Increasingly, third party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for migalastat HCl or any product that we commercialize and, if coverage and reimbursement are available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for migalastat HCl may be particularly difficult because of the higher prices



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typically associated with drugs directed at smaller populations of patients. In addition, third party payors are likely to impose strict requirements for reimbursement of a higher priced drug. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the EU, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. In addition, in the EU, for medicines authorized by the Centralised Authorisation Procedure, an authorized trader, such as a wholesaler, can purchase a medicine in one EU member state and import the product into another EU member state. This process is called parallel distribution. As a result, a purchaser in one EU member state may seek to import a product from another EU member state where such product is sold at a lower price. This could have a negative impact on our business, financial condition, results of operations and growth if any of our product candidates are approved in the EU.

***Any product or product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties or other enforcement actions if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product or our product candidates, when and if any of them are approved.***

Any product or product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. The FDA's requirements include submissions of safety and other post-marketing information and reports, registration requirements, Current Good Manufacturing Practices, or cGMP, requirements relating to manufacturing, quality control, quality assurance and complaints and corresponding maintenance of records and documents, requirements regarding the distribution of samples to healthcare professionals and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval, including the requirement of a REMS. The FDA also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not comply with the laws governing promotion of approved drugs, we may be subject to enforcement action for off-label promotion. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to civil and criminal penalties, investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;

- changes to or restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to implement a REMS;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;

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- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions; or
- the imposition of civil or criminal penalties.

Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the EU's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

If we, or our suppliers, third party contractors, clinical investigators or collaborators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or adoption of new regulatory requirements or policies, we or our collaborators may lose marketing approval for our products when and if any of them are approved, resulting in decreased revenue from milestones, product sales or royalties.

***Our relationships with customers, healthcare providers, patients, patient organizations and professionals and third party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and payors play a primary role in the recommendation and prescription of any product candidates for which we may obtain marketing approval. Our future arrangements with payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we may obtain marketing approval. Even though we do not and will not control referrals of

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healthcare services or bill directly to Medicare, Medicaid or other third party payors, federal, state and foreign healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal, state and foreign healthcare laws and regulations may affect our ability to operate and expose us to areas of risk, including:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse, and healthcare laws and regulations;
- the U.S. federal False Claims Act, which imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. There is also a separate false claims provision imposing criminal penalties. Applicable regulations of both the EMA and EU member states also impose liability for failing to comply with fraud and abuse laws or improperly using information obtained in the course of clinical trials with the EMA or other regulatory authorities;
- The U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute to defraud any healthcare benefit program or specific intent to violate it in order to have committed a violation. This statute also may impose monetary penalties on any offers or transfers of remuneration to Medicare or Medicaid beneficiaries (patients) which is likely to influence the beneficiary's selection of particular supplier of government payable items. Similarly, the collection and use of personal health data in the EU is governed by the provisions of the Data Protection Directive. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive also imposes strict rules on the transfer of personal data out of the EU to the United States. Failure to comply with the requirements of

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the Data Protection Directive as transposed into related national data protection laws of the EU member states may result in fines and other administrative penalties. The draft Data Protection Regulation (which, when adopted, does not require transposition into the national laws of the EU states) currently going through the legislative 10-decision process is expected to introduce new data protection requirements in the EU and substantial fines for breaches of the data protection rules. If the draft Data Protection Regulation is adopted in its current form it may increase our responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- U.S. federal laws requiring drug manufacturers to report annually information related to certain payments and other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership or investment interests held by physicians and their immediate family members, including under the federal Open Payments program, commonly known as the Sunshine Act, as well as other state and foreign laws regulating marketing activities and requiring manufacturers to report marketing expenditures, payments and other transfers of value to physicians and other healthcare providers. Similarly, payments made to physicians in certain EU member states must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU member states. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU member states. In addition, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the EU. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.
- U.S. federal government price reporting laws, which require us to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on our marketed drugs. Participation in these programs and compliance with the applicable requirements may subject us to potentially significant discounts on our products, increased infrastructure costs, potential liability for the failure to report such prices in an accurate and timely manner, and potentially limit our ability to offer certain marketplace discounts; and
- state and foreign equivalents of each of the above laws, including state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental payors, including private insurers; state laws which require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated

by the federal government or otherwise restricting payments that may be made to healthcare providers; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

While we do not submit claims and our customers will make the ultimate decision on how to submit claims, we may provide reimbursement guidance and support regarding migalastat HCl, and our product candidates for which we receive regulatory approval, to our customers and patients. If a government authority were to conclude that we provided improper advice to our customers and/or encouraged the submission of false claims for reimbursement, we could face action by government authorities. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Nonetheless, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations.

*Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.*

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of migalastat HCl or any of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates, including migalastat HCl, for which we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other

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provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the Affordable Care Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Effective October 1, 2010, the Affordable Care Act revised the definition of average manufacturer price for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. A significant number of provisions are not yet, or have only recently become, effective, but the Affordable Care Act is likely to continue the downward pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs. We expect that the Affordable Care Act, as well as other healthcare reform measures that have been and may be adopted in the future, may result in more rigorous coverage criteria, new payment methodologies and in additional downward pressure on the price that we receive for any approved product, and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may compromise our ability to generate revenue, attain profitability or commercialize our products.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

In the EU, similar political, economic and regulatory developments may affect our ability to profitably commercialize our products. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs.

***The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have promoted off-label uses, we may become subject to significant liability.***

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription drug products. In particular, a product may not be promoted in the United States for uses that are not approved by the FDA as reflected in the product's approved labeling. In particular, any labeling approved by the FDA for migalastat HCl, SD-101 or any of our other product candidates may include restrictions on use. The FDA may impose further requirements or restrictions on the distribution or use of migalastat HCl, SD-101 or any of our other product candidates as part of a REMS plan. If we receive marketing approval for migalastat HCl, SD-101 or any other product candidates, physicians may nevertheless prescribe such products to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines and / or other penalties against companies for alleged improper promotion and has investigated and / or prosecuted several companies in relation to off-label promotion (which is a violation of Federal regulations). The FDA has also requested that certain companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed, curtailed or prohibited.

*Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.*

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;



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- regulatory investigations, prosecutions or enforcement actions that could require costly recalls or product modifications
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- increased insurance costs, or an inability to maintain appropriate insurance coverage;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage for the commercialization of migalastat HCl and when, and if, we begin commercializing any product candidate that receives marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

*If the FDA or other applicable regulatory authorities approve generic products with claims that compete with our product or any of our product candidates, it could reduce our sales of our product or those product candidates.*

In the United States, after an NDA is approved, the product covered thereby becomes a listed drug which can, in turn, be cited by potential competitors in support of approval of an abbreviated NDA, or ANDA. The Federal Food, Drug, and Cosmetic Act, or the FD&C Act, FDA regulations and other applicable regulations and policies provide incentives to manufacturers to create modified, non-infringing versions of a

drug to facilitate the approval of an ANDA or other application for generic substitutes. These manufacturers might only be required to conduct a relatively inexpensive study to show that their product has the same active ingredients, dosage form, strength, route of administration, and conditions of use, or product labeling, as our product or product candidate and that the generic product is absorbed in the body at the same rate and to the same extent as, or is bioequivalent to, our product or product candidate. These generic equivalents would be significantly less costly than ours to bring to market and companies that produce generic equivalents are generally able to offer their products at lower prices. Thus, after the introduction of a generic competitor, a significant percentage of the sales of any branded product are typically lost to the generic product. Accordingly, competition from generic equivalents to our product or product candidates would substantially limit our ability to generate revenues and therefore to obtain a return on the investments we have made in our product or product candidates.

#### **Risks Related to our Intellectual Property**

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

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States and in certain foreign jurisdictions related to our novel technologies, product and product candidates that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, if we license technology or product candidates from third parties in the future, these license agreements may not permit us to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering this intellectual property. These agreements could also give our licensors the right to enforce the licensed patents without our involvement, or to decide not to enforce the patents at all. Therefore, in these circumstances, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value

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of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents issued to us or our licensors will provide a basis for commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- our patents will not expire prior to or shortly after commencing commercialization of a product; or
- the patents of others will not have a negative effect on our ability to do business.

In addition, we cannot be assured that any of our pending patent applications will result in issued patents. In particular, we have filed patent applications in the United States, the European Patent Office and other countries outside the United States that have not been issued as patents. These pending applications include, among others, some of the patent applications we license pursuant to a license agreement with Mount Sinai School of Medicine of New York University. If patents are not issued in respect of our pending patent applications, we may not be able to stop competitors from marketing similar products in Europe and other countries in which we do not have issued patents.

The patents that we have licensed from Mt. Sinai School of Medicine relating to use of migalastat HCl to treat Fabry disease expire in 2018 in the United States and 2019 in Europe, Japan, and Canada. In addition to patent protection outside of the United States, we intend to seek orphan medicinal product designation and to rely on statutory data exclusivity provisions in jurisdictions outside the United States where such protections are available, including Europe. The patent rights that we own or have licensed relating to our product candidates are limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. In particular:

- We do not hold composition of matter patents covering migalastat HCl. Composition of matter patents can provide protection for pharmaceutical products to the extent that the specifically covered compositions are important. For our product candidates for which we do not hold composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method of use patents that we may hold.
- For some of our product candidates, the principal patent protection that covers or those we expect will cover, our product candidate is a method of use patent. This type of patent only protects the product when used or sold for the specified method. However, this type of patent does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

Moreover, physicians may prescribe such a competitive identical product for indications other than the one for which the product has been approved, or off-label indications, that are covered by the applicable patents. Although such off-label prescriptions may infringe or induce infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. In addition, we may not pursue or obtain patent protection in all major markets. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

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Moreover, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office or become involved in opposition, derivation, reexamination, inter partes review, post grant review, interference proceedings or other patent office proceedings or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. In addition, other companies may attempt to circumvent any regulatory data protection or market exclusivity that we obtain under applicable legislation, which may require us to allocate significant resources to preventing such circumvention. Legal and regulatory developments in the EU and elsewhere may also result in clinical trial data submitted as part of an MAA becoming publicly available. Such developments could enable other companies to circumvent our intellectual property rights and use our clinical trial data to obtain marketing authorizations in the EU and in other jurisdictions. Such developments may also require us to allocate significant resources to prevent other companies from circumventing or violating our intellectual property rights. Our attempts to prevent third parties from circumventing our intellectual property and other rights may ultimately be unsuccessful. We may also fail to take the required actions or pay the necessary fees to maintain our patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file claims, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be accused of infringing one or more claims of an issued patent or may fall within the scope of one or more claims in a published patent application that may subsequently issue and to which we do not hold a license or other rights. Third parties may own or control these

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patents or patent applications in the United States and abroad. These third parties could bring claims against us that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our product candidates, technology or methods. Because of the number of patents issued and patent applications filed in our field, we believe there is a risk that third parties may allege they have patent rights encompassing our product candidates, technology or methods.

We are aware, for example, of U.S. patents, and corresponding international counterparts, owned by third parties that contain claims related to treating protein misfolding. If any of these patents were to be asserted against us, while we do not believe that our product candidates would be found to infringe any valid claim of such patents, there is no assurance that a court would find in our favor or that, if we choose or are required to

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seek a license with respect to such patents, such license would be available to us on acceptable terms or at all. If we were to challenge the validity of any issued U.S. patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity.

In order to avoid or settle potential claims with respect to any of the patent rights described above or any other patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly. Others may sue us for infringing their patent or other intellectual property rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. Furthermore, during the course of litigation, confidential information may be disclosed in the form of documents or testimony in connection with discovery requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. In addition to infringement claims against us, we may become a party to other patent litigation and other proceedings, including interference proceedings declared by the U.S. Patent and Trademark Office and opposition proceedings in the European Patent Office, regarding intellectual property rights with respect to our products and technology. Even if we prevail, the cost to us of any patent litigation or other proceeding could be substantial.

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 any litigation could significantly limit our ability to continue our operations. Patent litigation and other proceedings may also absorb significant management time.

***We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.***

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

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

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

***Intellectual property litigation could cause us to spend substantial resources and could distract our personnel from their normal responsibilities.***

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.



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***If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.***

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be obtained or independently developed by a competitor, our competitive position would be harmed.

***If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose license rights that are important to our business.***

We are a party to a license agreement with the Mount Sinai School of Medicine of New York University, pursuant to which we license key intellectual property relating to our lead product candidates. We expect to enter into additional licenses in the future. Under our existing license, we have the right to enforce the licensed patent rights. Our existing license imposes, and we expect that future licenses will impose, various diligences, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the right to terminate the license, in which event we might not be able to market any product that is covered by the licensed patents.

***We have not yet registered our trademarks in all of our potential markets, and failure to secure those registrations could adversely affect our business.***

Our trademark applications may not be allowed for registration, and our registered trademarks may not be maintained or enforced. During trademark registration proceedings, we may receive rejections. Although we are given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the U.S. Patent and Trademark Office and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we do not secure registrations for our trademarks, we may encounter more difficulty in enforcing them against third parties than we otherwise would.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

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The U.S. Patent and Trademark Office and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents are required to be paid to the U.S. Patent and Trademark Office and foreign patent agencies in several stages over the lifetime of the patents. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

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**Risks Related to our Dependence on Third Parties**

*Use of third parties to manufacture our product or product candidates may increase the risk that we will not have sufficient quantities of our product or product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.*

We do not own or operate manufacturing facilities for clinical or commercial production of our product or product candidates. We lack the resources and the capabilities to manufacture any of our product or product candidates on a clinical or commercial scale. We currently outsource all manufacturing and packaging of our product and preclinical and clinical product candidates to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields and quality control, including stability of the product or product candidate. The occurrence of any of these problems could significantly delay our clinical trials or the commercial availability of our products.

We may be unable to enter into agreements for commercial supply with third party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time.

Even if we are able to establish and maintain arrangements with third party manufacturers, reliance on third party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and

- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

The FDA and regulatory authorities in other jurisdictions require our contract manufacturers to comply with regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure or the failure of our third party manufacturers, to comply with applicable regulations could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

If the third parties that we engage to manufacture product for our preclinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that receive regulatory approval on a timely and competitive basis.

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***We rely on third parties to conduct certain preclinical development activities and our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.***

We do not independently conduct clinical trials for our product candidates or certain preclinical development activities of our product candidates, such as long-term safety studies in animals. We rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to perform these functions. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for preclinical and clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, or GCP, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. Similar GCP and transparency requirements apply in the EU. Failure to comply with such requirements, including with respect to clinical trials conducted outside the EU and United States, can also lead regulatory authorities to refuse to take into account clinical trial data submitted as part of an MAA.

Furthermore, third parties that we rely on for our clinical development activities may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Our product development costs will increase if we experience delays in testing or obtaining marketing approvals.

We also rely on other third parties to store and distribute drug supplies for our preclinical development activities and clinical trials. 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, producing additional losses and depriving us of potential product revenue.

Extensions, delays, suspensions or terminations of our preclinical development activities or our clinical trials as a result of the performance of our independent clinical investigators and CROs will delay, and make more costly, regulatory approval for any product candidates that we may develop. Any change in a CRO during an ongoing preclinical development activity or clinical trial could seriously delay that trial and potentially compromise the results of the activity or trial.

***We may not be successful in maintaining or establishing collaborations, which could adversely affect our ability to develop and, particularly in international markets, commercialize products.***

We are collaborating with physicians, patient advocacy groups, foundations and government agencies in order to assist with the development of our products and each of our product candidates. We plan to pursue similar activities in future programs and plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or

biotechnology companies. We also may seek to establish collaborations for the sales, marketing and distribution of our products. If we elect to seek collaborators in the future but are unable to reach agreements with suitable collaborators, we may fail to meet our business objectives for the affected product or program. We face, and will continue to face, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts, if any, to establish and implement collaborations or other alternative arrangements. The terms of any collaboration or other arrangements that we establish, if any, may not be favorable to us.

Any collaboration that we enter into may not be successful. The success of our collaboration arrangements, if any, will depend heavily on the efforts and activities of our collaborators. It is likely that any collaborators of ours will have significant discretion in determining the efforts and resources that they will apply to these collaborations. The risks that we may be subject to in possible future collaborations include the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product or product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

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- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;
- we may grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

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Collaborations with pharmaceutical companies and other third parties often are terminated or allowed to expire by the other party. Such terminations or expirations may adversely affect us financially and could harm our business reputation in the event we elect to pursue collaborations that ultimately expire or are terminated.

Our initial co-formulated product candidate for Fabry Disease that we developed as part of our collaboration with GSK utilized migalastat HCl co-formulated with a proprietary human recombinant alpha-Gal A enzyme. We plan to continue development of a co-formulated ERT with migalastat HCl with an internally developed Fabry cell line as a next-generation ERT for Fabry disease.

The risks involved with developing our own internal cell line are in addition to the risks described above with respect to securing and using third party manufacturers and it could significantly and adversely affect the overall cost of developing the co-formulated product candidate and significantly increase the timelines for development.

***Materials necessary to manufacture our product or product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our product or product candidates.***

We rely on the manufacturers of our product and product candidates to purchase from third party suppliers the materials necessary to produce the compounds for our preclinical studies and clinical trials, and we rely, or will rely, on these other manufacturers for commercial distribution of our product and, if we obtain marketing approval, for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical studies and clinical trials, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop and commercialize our product candidates. If our manufacturers or we are unable to purchase these materials for commercial distribution of our product or, after regulatory approval has been obtained, our product candidates, the commercial launch of our product and product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product or product candidates.



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***Manufacturing issues may arise that could increase product and regulatory approval costs or delay commercialization.***

As we scale up manufacturing of our product and product candidates and conduct required stability testing, we may encounter product, packaging, equipment and process-related issues that may require refinement or resolution in order to proceed with our planned clinical trials and obtain regulatory approval for commercial marketing. In the future, we may identify impurities, which could result in increased scrutiny by regulatory authorities, delays in our clinical programs and regulatory approval, increases in our operating expenses or failure to obtain or maintain approval for our product or product candidates.

***Our business activities involve the use of hazardous materials, which require compliance with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.***

Our research and development programs involve the controlled use of hazardous materials, including microbial agents, corrosive, explosive and flammable chemicals and other hazardous compounds in addition to certain biological hazardous waste. Ultimately, the activities of our third party product manufacturers when a product candidate reaches commercialization will also require the use of hazardous materials. Accordingly, we are subject to federal, state and local laws governing the use, handling and disposal of these materials. Although we believe that our safety procedures for handling and disposing of these materials comply in all material respects with the standards prescribed by local, state and federal regulations, we cannot completely eliminate the risk of accidental contamination or injury from these materials. In addition, our collaborators may not comply with these laws. In the event of an accident or failure to comply with environmental laws, we could be held liable for damages that result, and any such liability could exceed our assets and resources or we could be subject to limitations or stoppages related to our use of these materials which may lead to an interruption of our business operations or those of our third party contractors. While we believe that our existing insurance coverage is generally adequate for our normal handling of these hazardous materials, it may not be sufficient to cover pollution conditions or other extraordinary or unanticipated events. Furthermore, an accident could damage or force us to shut down our operations. Changes in environmental laws may impose costly compliance requirements on us or otherwise subject us to future liabilities and additional laws relating to the management, handling, generation, manufacture, transportation, storage, use and disposal of materials used in or generated by the manufacture of our products or related to our clinical trials. In addition, we cannot predict the effect that these potential requirements may have on us, our suppliers and contractors or our customers.

**Risks Related to our Business, Employee Matters and Managing Growth**

***Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.***

We are highly dependent on John F. Crowley, our Chairman and Chief Executive Officer, Bradley L. Campbell, our President and Chief Operating Officer, William D. Baird, III, our Chief Financial Officer and Jay Barth, M.D., our Chief Medical Officer. These executives each have significant pharmaceutical industry experience. The loss of the services of any of these executives might impede the achievement of our research, development and commercialization objectives and materially adversely affect our business. We do not maintain key person insurance on Mr. Crowley or on any of our other executive officers.

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Recruiting and retaining qualified scientific, clinical and sales and marketing personnel will also be critical to our success. In addition, maintaining a qualified finance and legal department is key to our ability to meet our regulatory obligations as a public company and important in any potential capital raising activities. Our industry has experienced a high rate of turnover in recent years. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel, particularly in New Jersey and surrounding areas. Although we believe we offer competitive salaries and benefits, we may have to increase spending in order to retain personnel. If we fail to retain our remaining qualified personnel or replace them when they leave, we may be unable to continue our development and commercialization activities.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

***We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

As of September 30, 2016, we had 241 full-time employees. As our development and commercialization strategies develop, we will need additional managerial, operational, sales, marketing, financial and other resources. Our management, personnel and systems currently in place may

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not be adequate to support this future growth. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Future growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of our existing or future product candidates. Future growth would impose significant added responsibilities on members of management, including:

- managing the commercialization of migalastat HCl and any product candidates approved for marketing;
- overseeing our ongoing preclinical studies and clinical trials effectively;
- identifying, recruiting, maintaining, motivating and integrating additional employees, including any sales and marketing personnel engaged in connection with the commercialization of any approved product;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- improving our managerial, development, operational and financial systems and procedures;
- developing our compliance infrastructure and processes to ensure compliance with regulations applicable to public companies; and
- expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic collaborators, suppliers and other third parties. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing our company.

*We could be negatively impacted by securities class action complaints.*

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Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for the District of New Jersey, naming us as defendants, along with our Chief Executive Officer and, in one of the actions, our Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934, as amended, or the Exchange Act, in connection with allegedly false and misleading statements made by us related to the regulatory approval path for migalastat HCl. The plaintiffs seek, among other things, damages for purchases of our common stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming us and/or our officers and directors as defendants. These lawsuits have been combined into a consolidated action. This action and any other related lawsuits are subject to inherent uncertainties, and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain and we could be forced to expend significant resources in the defense of this suit, and we may not prevail. We are not currently able to estimate the possible cost to us from this matter, as this lawsuit is currently at an early stage and we cannot ascertain how long it may take to resolve this matter. Moreover, such litigation may impact our ability to raise future capital, which could negatively impact our product candidate development and commercialization efforts.

On or about November 2, 2015, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division. Defendants are the individuals who serve on the Amicus Board of Directors. Amicus itself is named as a nominal defendant. Filed shortly after three purported securities class action lawsuits filed in the District of New Jersey, the derivative lawsuit alleges claims for breach of state law fiduciary duties, waste of corporate assets, and unjust enrichment based on alleged violations of the Securities Exchange Act of 1934, in connection with allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. On February 16, 2016, the complaint was dismissed by the Court and plaintiffs have not refiled.

On or about March 3, 2016, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against various officers and directors of the Company. Amicus itself is named as a nominal defendant. The derivative lawsuit alleges similar facts and circumstances as the three purported securities class action lawsuits described above and further alleges claims for breach of state law fiduciary duties, waste of corporate assets, unjust enrichment, abuse of control, and gross mismanagement based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. The plaintiff seeks, among other things, to require the Amicus Board to take certain actions to reform its corporate governance procedures, including greater shareholder input and a provision to permit shareholders to nominate candidates for election to the Board, along with restitution, costs of suit and attorney's fees. The parties have entered into a stipulation to stay the time to respond to the derivative complaint until resolution of any motion to dismiss in the above-referenced securities action.

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This action and any other related lawsuits are subject to inherent uncertainties, and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain and we could be forced to expend significant resources in the defense of this suit, and we may not prevail. We are not currently able to estimate the possible cost to us from this matter, as this lawsuit is currently at an early stage and we cannot ascertain how long it may take to resolve this matter. Moreover, such litigation may impact our ability to raise future capital, which could negatively impact our product candidate development and commercialization efforts.

***Our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.***

We are exposed to the risk that our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates:

- FDA, DEA or similar regulations of foreign regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities;
- manufacturing standards;
- federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by foreign regulatory authorities; or
- laws that require the reporting of financial information or data accurately.

In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct and Ethics, but it is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a material adverse effect on our business and results of operations, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could have a material adverse effect on our ability to operate our business and our results of operations.

*Our business and operations would suffer in the event of computer system failures.*

Despite the implementation of security measures, our internal computer systems, and those of our CROs, contract manufacturing organizations and other third parties on which we rely, we are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our commercialization of our product and our product candidate development programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruptions or security breach was to result in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

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**Risks Related to our Common Stock**

*Our executive officers, directors and principal stockholders maintain the ability to exert significant influence and control over matters submitted to our stockholders for approval.*

Our executive officers, directors and affiliated stockholders beneficially own shares representing approximately 13% of our common stock as of December 31, 2015. As a result, if these stockholders were to choose to act together, they would be able to exert significant influence and control over matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could influence the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with the interests of other stockholders, and they may act, whether by meeting or written consent of stockholders, in a manner that advances their best interests and not necessarily those of other stockholders, including obtaining a premium value for their common stock, and might affect the prevailing market price for our common stock.

*Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.*

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Because our board of directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. Among others, these provisions:

- establish a classified board of directors, and, as a result, not all directors are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock, without stockholder approval, which could be used to institute a poison pill that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 67% of the outstanding voting stock to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

*The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.*

The market price of our common stock is highly volatile and may be subject to wide fluctuations in response to various factors, some of which are beyond our control. In addition to the factors discussed in this prospectus, these factors include:

- the success of competitive products or technologies;



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- regulatory actions with respect to our product or product candidates or our competitors' products or product candidates;
- actual or anticipated changes in our growth rate relative to our competitors;
- the outcome of any patent infringement or other litigation that may be brought against us;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, collaborations or capital commitments;
- results of clinical trials of our product candidates or those of our competitors;
- regulatory or legal developments in the EU, United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to our product or any of our product candidates or clinical development programs;
- actual or anticipated variations in our quarterly operating results;
- the number and characteristics of our efforts to in-license or acquire additional product candidates or products;

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- introduction of new products or services by us or our competitors;
- failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- changes in accounting practices;
- significant lawsuits, including patent or stockholder litigation, including the three purported class action lawsuits that have been brought against us in the U.S. District Court for the District of New Jersey;
- other lawsuits, including a shareholder derivative action which has been brought against our officers and Directors on behalf of the Company in the Superior Court of New Jersey, Middlesex County
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;

- general economic, industry and market conditions;
- publication of research reports about us, our competitors or our industry, or positive or negative recommendations or withdrawal of research coverage by securities or industry analysts;
- other events or factors, many of which are beyond our control; and
- the other factors described in this Risk Factors section.

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In addition, the stock market in general, and pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks stated above could have a material adverse effect on the market price of our common stock.

As we operate in the pharmaceutical and biotechnology industry, we are especially vulnerable to these factors to the extent that they affect our industry or our products. In the past, securities class action litigation has often been initiated against companies following periods of volatility in their stock price. This type of litigation could result in substantial costs and divert our management's attention and resources, and could also require us to make substantial payments to satisfy judgments or to settle litigation.

***A significant portion of our total outstanding shares may be sold into the market. This could cause the market price of our common stock to drop significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Certain holders of our common stock have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also have registered on a Form S-8 registration statement all shares of common stock that we may issue under our equity compensation plans. As a result, these shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. In addition, certain of our employees, executive officers and directors have entered into, or may enter into, Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the employee, director or officer when entering into the plan, without further direction from the employee, officer or director. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers and directors may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

***We may fail to qualify for continued listing on The NASDAQ Global Market which could make it more difficult for investors to sell their shares.***

Our common stock is listed on The NASDAQ Global Market, or NASDAQ. As a NASDAQ listed company, we are required to satisfy the continued listing requirements of NASDAQ for inclusion in the Global Market to maintain such listing, including, among other things, the maintenance of a minimum closing bid price of \$1.00 per share and stockholders' equity of at least \$10.0 million. There can be no assurance that we will be able to maintain compliance with the continued listing requirements or that our common stock will not be delisted from NASDAQ in the future. If our common stock is delisted by NASDAQ, we could face significant material adverse consequences, including:

- a limited availability of market quotations for our securities;
- reduced liquidity with respect to our securities;

- a determination that our shares are a penny stock, which will require brokers trading in our shares to adhere to more stringent rules, possibly resulting in a reduced level of trading activity in the secondary trading market for our shares;
- a limited amount of news and analyst coverage for our company; and
- a decreased ability to issue additional securities or obtain additional financing in the future.

*If securities or industry analysts do not publish research or reports or publish unfavorable research about our business, the price of our common stock and trading volume could decline.*

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. If securities or industry analysts do not initiate or continue coverage of us, the trading price for our common stock would be negatively affected. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our common stock, the price of our common stock would likely decline. If one or more of these analysts ceases to cover us or fails to publish regular reports on us, interest in the purchase of our common stock could decrease, which could cause the price of our common stock or trading volume to decline.

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***We have broad discretion in the use of our cash and cash equivalents and may not use them effectively.***

We have broad discretion in the use of our cash and cash equivalents, and investors must rely on the judgment of our management regarding the use of our cash and cash equivalents. Our management may not use cash and cash equivalents in ways that ultimately increase the value of your investment. Our failure to use our cash and cash equivalents effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product and product candidates. Pending their use, we may invest our cash and cash equivalents in short-term or long-term, investment-grade, interest-bearing securities. These investments may not yield favorable returns. If we do not invest or apply our cash and cash equivalents in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause the price of our common stock to decline.

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

We are subject to the periodic reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the Securities and Exchange Commission. We believe that any disclosure controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations reflect the reality that judgments can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

***If the common stock issued as consideration in our recent acquisitions is sold, such sales could cause our common stock price to decline.***

The issuance of our common stock in connection with the Scioderm and MiaMed mergers could have the effect of depressing the market price for our common stock, through dilution of earnings per share or otherwise. All of the shares of common stock issued to the former security holders of Scioderm and MiaMed in connection with the closings of the mergers have been registered under the Securities Act of 1933, as amended, pursuant to automatic shelf registration statements on Form S-3 (File Nos. 333-207210 and 333-207210, as applicable) and may now be resold by the former security holders of Scioderm and MiaMed to investors in the general market.

***Because we do not anticipate paying any cash dividends on our capital in the foreseeable future, capital appreciation, if any, will be our stockholders sole source of gain.***

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders sole source of gain for the foreseeable future.



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**ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**

**Recent Sales of Unregistered Securities**

None.

**Issuer Purchases of Equity Securities**

The following table sets forth purchases of our Common Stock for the three months ended September 30, 2016:

<b>Period</b>	<b>(a) Total number of shares purchased</b>	<b>(b) Average Price Paid per Share</b>	<b>(c) Total number of shares purchased as part of publicly announced plans or programs</b>	<b>(d) Maximum number of shares that may yet be purchased under the plans or programs</b>
July 1, 2016 through August 31, 2016	10,695	6.37		23,878

There were no purchases of our Common Stock during the period September 1, 2016 to September 30, 2016.

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**ITEM 4. MINE SAFETY DISCLOSURES**

Not applicable.

**ITEM 5. OTHER INFORMATION**



None.

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**ITEM 6. EXHIBITS**

<b>Exhibit Number</b>	<b>Description</b>
3.1(1)	Restated Certificate of Incorporation
3.2(2)	Certificate of Amendment to the Company's Restated Certificate of Incorporation, as amended
3.3 (3)	Amended and Restated By-laws
10.1 (4)	Note and Warrant Purchase Agreement by and among Amicus Therapeutics, Inc., Amicus Therapeutics UK Limited and the purchasers identified on the signature pages thereto, dated as of February 19, 2016.
10.2 (5)	Joinder to and Amendment of Note and Warrant Purchase Agreement by and among Amicus Therapeutics, Inc., Amicus Therapeutics UK Limited, Amicus Therapeutics International Holding LTD and the purchasers identified on the signature pages thereto, dated as of June 30, 2016
10.3 (6)	Amendment No. 1 to the Amended and Restated Amicus Therapeutics, Inc. 2007 Equity Incentive Plan
10.4 (7)	Second Letter, dated August 22, 2016 by and between Amicus Therapeutics, Inc. and Bradley Campbell
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101	The following financial information from this Quarterly Report on Form 10-Q for the nine months ended September 30, 2016, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements

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- (1) Incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on August 5, 2015.
  - (2) Incorporated by reference to Exhibit 3.2 to our Quarterly Report on Form 10-Q filed on August 5, 2015.
  - (3) Incorporated by reference to Exhibit 3.3 to our Registration Statement on Form S-1.
  - (4) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on February 22, 2016.
  - (5) Incorporated by reference to Exhibit 10.2 to our Current Report on Form 8-K filed on July 1, 2016.
  - (6) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on July 29, 2016.
  - (7) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on July 29, 2016.



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**SIGNATURES**

Pursuant to the requirements of the Securities and Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AMICUS THERAPEUTICS, INC.

Date: November 7, 2016

By:

/s/ John F. Crowley  
John F. Crowley  
Chairman and Chief Executive Officer  
(Principal Executive Officer)

Date: November 7, 2016

By:

/s/ William D. Baird III  
William D. Baird III  
Chief Financial Officer  
(Principal Financial Officer)

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<b>Exhibit Number</b>	<b>Description</b>
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
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