

AMICUS THERAPEUTICS INC  
Form 10-Q  
August 09, 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 June 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.**

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(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

Accelerated filer

Non-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 July 29, 2016 was 142,139,451 shares.

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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 to identify 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;

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- the extent to which we acquire or invest in businesses, products and technologies;
- our ability to successfully integrate our recent acquisition of Scioderm, Inc. ( 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.

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)**

	<b>June 30, 2016</b>	<b>December 31, 2015</b>
<b>Assets:</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 63,656	\$ 69,485
Investments in marketable securities	150,494	144,548
Inventories	194	
Prepaid expenses and other current assets	3,330	2,568
<b>Total current assets</b>	<b>217,674</b>	<b>216,601</b>
Property and equipment, less accumulated depreciation of \$14,284 and \$13,353 at June 30, 2016 and December 31, 2015, respectively	10,178	6,178
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	1,657	1,108
<b>Total Assets</b>	<b>\$ 914,006</b>	<b>\$ 908,384</b>
<b>Liabilities and Stockholders Equity</b>		
<b>Current liabilities:</b>		
Accounts payable and accrued expenses	\$ 23,828	\$ 32,216
Contingent consideration payable, current portion	56,000	41,400
Other current liabilities	631	
<b>Total current liabilities</b>	<b>80,459</b>	<b>73,616</b>
Deferred reimbursements	35,756	35,756
Due to related party	43,443	41,601
Unsecured notes payable	21,851	
Contingent consideration payable, less current portion	220,300	232,677
Deferred tax liability	176,219	176,219
Other non-current liabilities	1,735	681
<b>Commitments and contingencies</b>		
<b>Stockholders equity:</b>		
Common stock, \$.01 par value, 250,000,000 authorized, 134,408,526 shares issued and outstanding at June 30, 2016, 250,000,000 shares authorized, 125,027,034 shares issued and outstanding at December 31, 2015	1,399	1,306
Additional paid-in capital	990,032	917,454
<b>Accumulated other comprehensive loss:</b>		
Foreign currency translation adjustment, less tax benefit of \$453 at June 30, 2016	842	

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Unrealized gain/ (loss) on available-for securities	201	(115)
Warrants	16,076	8,755
Accumulated deficit	(674,307)	(579,566)
Total stockholders' equity	334,243	347,834
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 914,006</b>	<b>\$ 908,384</b>

*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 June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
<b>Operating Expenses:</b>				
Research and development	\$ 18,281	\$ 17,234	\$ 41,706	\$ 33,347
General and administrative	19,300	8,278	35,001	14,705
Changes in fair value of contingent consideration payable	10,186	100	13,338	1,100
Restructuring charges	8	26	58	36
Loss on extinguishment of debt		952		952
Depreciation	767	353	1,440	861
Total operating expenses	48,542	26,943	91,543	51,001
Loss from operations	(48,542)	(26,943)	(91,543)	(51,001)
<b>Other income (expenses):</b>				
Interest income	331	158	638	329
Interest expense	(1,055)	(338)	(2,000)	(710)
Other expense	(2,237)	(10)	(2,289)	(39)
Loss before income tax benefit	(51,503)	(27,133)	(95,194)	(51,421)
Income tax benefit	453		453	
Net loss	(51,050)	(27,133)	(94,741)	(51,421)
Net loss per common shares basic and diluted	\$ (0.40)	\$ (0.27)	\$ (0.75)	\$ (0.53)
Weighted-average common shares outstanding basic and diluted	129,122,175	99,994,125	127,160,943	97,888,573

*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 June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2016</b>	<b>2015</b>	<b>2016</b>	<b>2015</b>
Net loss	\$ (51,050)	\$ (27,133)	\$ (94,741)	\$ (51,421)
Other comprehensive gain /(loss)				
Foreign currency translation adjustment, net of tax \$453	907		842	
Unrealized gain/(loss) on available- for-sale securities	87	(17)	316	80
Other comprehensive gain /(loss)	\$ 994	\$ (17)	\$ 1,158	\$ 80
Comprehensive loss	\$ (50,056)	\$ (27,150)	\$ (93,583)	\$ (51,341)

*See accompanying notes to consolidated financial statements*

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

(in thousands)

	Six Months Ended June 30,	
	2016	2015
<b>Operating activities</b>		
Net loss	\$ (94,741)	\$ (51,421)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	1,014	136
Depreciation	1,440	861
Stock-based compensation	8,748	4,191
Restructuring charges	58	36
Loss on extinguishment of debt		952
Loss on disposal of asset	17	
Non-cash changes in the fair value of derivative liability	346	
Non-cash changes in the fair value of contingent consideration payable	13,338	1,100
Foreign currency remeasurement loss	1,892	
Non-cash income tax benefit	(453)	
Changes in operating assets and liabilities:		
Inventories	(207)	
Prepaid expenses and other current assets	(865)	641
Other non-current assets	(549)	(482)
Accounts payable and accrued expenses	(8,244)	459
Non-current liabilities	535	(84)
Net cash used in operating activities	(77,671)	(43,611)
<b>Investing activities</b>		
Sale and redemption of marketable securities	121,283	63,163
Purchases of marketable securities	(126,914)	(30,414)
Purchases of property and equipment	(4,608)	(1,429)
Net cash (used in)/ provided by investing activities	(10,239)	31,320
<b>Financing activities</b>		
Proceeds from issuance of common stock, net of issuance costs	57,818	243,216
Proceeds from unsecured note agreement	30,000	
Payments of secured loan agreement		(15,291)
Payment of capital lease	(47)	
Payment of contingent consideration	(5,000)	
Proceeds from exercise of stock options	647	6,932
Purchase of vested restricted stock units	(657)	(1,617)
Proceeds from exercise of warrants		4,000
Net cash provided by financing activities	82,761	237,240
Effect of exchange rate changes on cash and cash equivalents	(680)	
Net (decrease)/ increase in cash and cash equivalents	(5,829)	224,949
Cash and cash equivalents at beginning of period	69,485	24,074
Cash and cash equivalents at end of period	\$ 63,656	\$ 249,023
<b>Supplemental disclosures of cash flow information</b>		
Cash paid during the period for interest	\$ 276	\$ 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 Consolidated Financial Statements**

**Note 1. Description of Business**

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

Amicus Therapeutics, Inc. (the Company, we, us, or our ) was incorporated on February 4, 2002 in Delaware and 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 candidate, 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. SD-101, a product candidate in late-stage development, is 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.

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 has 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 began supplying the market in Germany in May 2016 and will commence the reimbursement processes with healthcare authorities in each of the major European countries.

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.

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 GCM 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.

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In February 2016, the Company entered into a sales agreement ( Sales Agreement ) with Cowen and Company, LLC ( Cowen ), to create an at-the-market ( ATM ) equity program under which the Company from time to time may offer and sell shares of its Common Stock having an aggregate offering price of up to \$100.0 million through Cowen as sales agent for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate up to 3.0% of the gross proceeds per share sold through it as sales agent under the sales agreement. Beginning in April 2016 and through June 30, 2016, the Company sold 8.2 million shares of Common Stock under the ATM sales agreement resulting in net proceeds of \$57.8 million, after Cowen s commission of \$1.7 million and other expenses of \$0.1 million. In July 2016, the Company sold an additional 6.8 million shares of Common Stock with a net proceeds of \$39.3 million. In connection with the July share sales, the Company completed all sales under the ATM equity program.

For more details, refer to Note 13. Subsequent Events.

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The Company had an accumulated deficit of approximately \$674.3 million at June 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 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 expects to recognize revenue 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 second half 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.



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***Significant Accounting Policies***

There have been no material changes to the Company's significant accounting policies during the six months ended June 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.

***Inventories***

Until regulatory approval of migalastat, the Company expensed all of its inventory costs as research and development expense. Upon regulatory approval, the Company began capitalizing costs related to the purchase and manufacture of migalastat. Inventories are stated at the lower of cost or market determined by the first-in, first-out method. The Company will analyze its inventory levels quarterly and will write down inventory that has become obsolete, or has a cost basis in excess of its expected net realizable value and inventory quantities in excess of expected requirements. Expired inventory will be disposed of and the related costs will be recognized as cost of goods sold in the Company's Consolidated Statements of Operations.

***Revenue Recognition***

***Net Product Sales***

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

The Company will record revenue on sales where migalastat is available on a reimbursed expanded access program and typically paid for by a government authority or institution. The Company will recognize revenue for these reimbursed expanded access programs on a cash basis if all other revenue recognition criteria have been met. Once the Company has established a pattern of collectability, revenue will be recognized upon shipment assuming all other revenue recognition criteria are met.

***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

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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.

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.

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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.

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.

***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 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

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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 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 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. 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 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. For private companies, the amendments are effective for annual periods beginning after December 15, 2017, and interim periods within annual periods beginning after December 15, 2018. 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

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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 is currently assessing the impact that this standard will have on its consolidated financial statements.

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.

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

As of June 30, 2016, the Company held \$63.7 million in cash and cash equivalents and \$150.5 million of 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 six months ended June 30, 2016, the Company recognized a loss of \$346 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 \$346 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 June 30, 2016 and December 31, 2015 (in thousands):

	As of June 30, 2016			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	\$ 63,656	\$	\$	\$ 63,656
Corporate debt securities	85,475	\$ 19	\$ (23)	\$ 85,471
Commercial paper	64,468	\$ 205		\$ 64,673
Certificate of deposit	350			350
	\$ 213,949	\$ 224	\$ (23)	\$ 214,150
Included in cash and cash equivalents	\$ 63,656	\$	\$	\$ 63,656
Included in marketable securities	\$ 150,293	\$ 224	\$ (23)	\$ 150,494
Total cash and marketable securities	\$ 213,949	\$ 224	\$ (23)	\$ 214,150

	As of December 31, 2015			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	\$ 69,485	\$	\$	\$ 69,485
Corporate debt securities	118,627	1	(154)	118,474

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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



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For the six months ended June 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 June 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 \$50.4 million and \$118.5 million as of June 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 raw materials, work in process and finished goods related to the manufacture of Galafold. The following table summarizes the components of inventories at June 30, 2016 (in thousands):

(Dollars in thousands)	June 30, 2016	
Raw materials	\$	
Work-in-process		137
Finished goods		57
<b>Total inventories</b>	<b>\$</b>	<b>194</b>

There were no inventories expected to remain on-hand beyond one year at June 30, 2016, and there were no inventories on hand at December 31, 2015.

**Note 5. Acquisitions****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*.

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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 ( FDSIA ) and the Company will request a Priority Review Voucher ( PRV ) under the FDSIA, 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.

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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 anticipated approval assumptions as of June 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 \$265.8 million at June 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 \$13.0 million for the six months ended June 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.

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 June 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 \$10.5 million at June 30, 2016, of which \$10.1 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 three months ended June 30, 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 three months ended June 30, 2016, resulting in \$6.1 million impact on stockholder's equity.

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

Table of Contents**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 six months ended June 30, 2016:

	( in millions)	
Balance at December 31, 2015	\$	197.8
Change in goodwill		
Balance at June 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 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 six months ended June 30, 2016:

	( in millions)	
Balance at December 31, 2015	\$	486.7
Change in IPR&D		
Balance at June 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 six months ended June 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 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.

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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. As of June 30, 2016, 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

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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 June 30, 2016 between Redmile and GCM as of June 30, 2016 is as follows (in thousands):

Creditor	Gross amount of debt	Net unamortized discount	Net carrying value of debt
RedMile	\$ 55,000	\$ (11,557)	\$ 43,443
GCM	25,000	(3,149)	21,851
Total Debt	\$ 80,000	\$ (14,706)	\$ 65,294

The debt discount amortization for the three and six months ended June 30, 2016 was \$0.6 million and \$1.0 million, respectively.

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

**Note 8. Stockholders' Equity****Common Stock and Warrants**

As of June 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.

In February 2016, the Company entered into the Sales Agreement with Cowen to create an at-the-market equity program under which the Company from time to time may offer and sell shares of its Common Stock, having an aggregate offering price of up to \$100 million through Cowen (the ATM Facility). Sales of the shares under the Sales Agreement were to be made in transactions that were deemed to be at the market offerings as defined in Rule 415 promulgated under the Securities Act of 1933, as amended, including sales made directly on The NASDAQ Global Market, on any other existing trading market for the Common Stock or to or through a market maker. In addition, with the Company's prior written approval, Cowen may also sell shares of Common Stock by any other method permitted by law, including in negotiated

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transactions. Cowen will act as sales agent using its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of The NASDAQ Stock Market LLC. There is no arrangement for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate up to 3.0% of the gross proceeds per share sold through it as sales agent under the sales agreement. Beginning in April 2016 and through June 30, 2016, the Company sold 8.2 million shares of Common Stock under the ATM sales agreement resulting in net proceeds of \$57.8 million, after Cowen's commission of \$1.7 million and other expenses of \$0.1 million. In July 2016, the Company sold an additional 6.8 million shares of Common Stock with net proceeds of \$39.3 million after Cowen's commission. In connection with the July share sales, the Company completed all sales under the ATM equity program.

In October 2015, the Company entered into the October 2015 Purchase Agreement with Redmile, whereby the Company sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its Notes and (b) Warrants for 1.3 million shares of Common Stock. On February 19, 2016, the Company entered into the February 2016 Purchase Agreement with Redmile for \$50.0 million in unsecured promissory notes and five-year warrants for 1.9 million shares of Common Stock. The Company agreed with Redmile to cancel the \$50 million note and warrants issued in October 2015 and pay only the accrued interest due of \$0.8 million. In accordance with ASC 470, the transactions qualified as a modification of debt.

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. Such amendment joined GCM to the February 2016 Purchase Agreement. Pursuant to the Amended Purchase Agreement, the Company sold an additional \$30.0 million unsecured



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promissory notes and five-year warrants to purchase up 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 issuable under the Additional Warrants. The 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 total outstanding warrants as of June 30, 2016 is as follows (in thousands):

<b>Creditor</b>	<b>Warrant shares</b>	<b>Warrant amount</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 June 30, 2016 on the Consolidated Balance Sheet.

***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.

Deferred compensation amounts under the Deferral Plan as of June 30, 2016 were approximately \$1.2 million, as compared to \$0.7 million on December 31, 2015 and are included in other long-term liabilities. Deferral Plan assets as of June 30, 2016 were \$1.2 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 and unrealized gain (loss) for the three and six months ended June 30, 2016 and 2015 were de minimis.

***Equity Incentive Plan***

*Stock Option Grants*

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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 June 30,		Six months ended June 30,	
	2016	2015	2016	2015
Expected stock price volatility	81.3%	74.3%	81.2%	75.6%
Risk free interest rate	1.3%	1.7%	1.7%	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

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A summary of the Company's stock options for the six months ended June 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	2,932.5	\$ 8.60		
Options exercised	(165.8)	\$ 3.90		
Options forfeited	(224.5)	\$ 8.78		
Balance at June 30, 2016	14,271.4	\$ 7.42	7.4 years	\$ 11.2
Vested and unvested expected to vest June 30, 2016	13,363.2	\$ 7.31	7.3 years	\$ 10.9
Exercisable at June 30, 2016	7,049.4	\$ 6.24	6.0 years	\$ 7.2

As of June 30, 2016, the total unrecognized compensation cost related to non-vested stock options granted was \$32.7 million and is expected to be recognized over a weighted average period of 3 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 six months ended June 30, 2016 is as follows:

	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	25.0	\$ 8.61		
Vested	(181.3)	\$ 6.55		
Forfeited		\$		
Non-vested units as of June 30, 2016	322.2	\$ 12.40	1.88	\$
Non-vested units expected to vest at June 30, 2016	322.2	\$ 12.40	1.88	\$

For the six months ended June 30, 2016, 181,250 of the RSUs vested and all non-vested units are expected to vest over their normal term.

As of June 30, 2016, there was \$2.8 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 1.85 year.

*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 June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Equity compensation expense recognized in:				
Research and development expense	\$ 1,966	\$ 1,044	\$ 3,902	\$ 1,991
General and administrative expense	2,500	1,187	4,846	2,200
Total equity compensation expense	\$ 4,466	\$ 2,231	\$ 8,748	\$ 4,191

Table of Contents**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 June 30, 2016, are identified in the following table (in thousands):

	Level 1	Level 2	Total
<b>Assets:</b>			
Cash/ money market funds	\$ 63,656	\$	\$ 63,656
Corporate debt securities		\$ 85,471	\$ 85,471
Commercial paper		\$ 64,673	\$ 64,673
Certificate of deposit		\$ 350	\$ 350
Market exchanged mutual funds		\$ 1,196	\$ 1,196
	\$ 63,656	\$ 151,690	\$ 215,346

	Level 2	Level 3	Total
<b>Liabilities:</b>			
Contingent consideration payable		\$ 276,300	\$ 276,300
Derivative liability	346		346
Deferred compensation plan liability	\$ 1,196		\$ 1,196
	\$ 1,542	\$ 276,300	\$ 277,842

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
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<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

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***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 six months ended June 30, 2016. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the six months ended June 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 \$65.3 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 June 30, 2016, the warrants are recorded at \$16.1 million and the notes at \$65.3 million, net of discount of \$14.7 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.

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

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Contingent Consideration Liability	Fair value as of June 30, 2016	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	\$ 239.8 million	Probability weighted discounted cash flow	Discount rate	0.4%-3.2%
			Probability of achievement of milestones	66.5% -100.0%
			Projected year of payments	2016-2018
Revenue-based milestones	\$ 26.0 million	Monte Carlo	Revenue volatility	58%
			Discount rate	0.6%-1.6%
			Projected year of payments	2018-2028



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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 June 30, 2016	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	\$ 10.1 million	Probability weighted discounted cash flow	Discount rate	10.5%
			Probability of achievement of milestones	30%-43%
			Projected year of payments	2018-2021

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 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.

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

	Three months ended June 30,		Six months ended June 30,	
	2016	2015	2016	2015
Balance, beginning of the period	\$ 277,229	\$ 11,700	\$ 274,077	\$ 10,700
Payment of contingent consideration in cash	(5,000)		(5,000)	
Payment of contingent consideration in stock	(6,115)		(6,115)	
Change in fair value change during the period, included in Statement of Operations	10,186	100	13,338	1,100
Balance, end of the period	\$ 276,300	\$ 11,800	\$ 276,300	\$ 11,800

*Deferred Compensation Plan- Investment and Liability*

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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 May 2017.

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

For the three and six months ended June 30, 2016, the Company recognized a loss of \$346 thousand related to the derivative instruments not designated as hedging instruments in the Consolidated Statements of Operations and the corresponding liability of \$346 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 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.

**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 six months ended June 30, 2016 (in thousands):

	Balance as of December 31, 2015	Charges	Cash Payments	Fair Value Adjustments	Balance as of June 30, 2016
Facilities consolidation	\$ 118	\$	\$ (135)	\$ 58	\$ 41

**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:

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(In thousands, except per share amounts)	Three months Ended June 30,		Six months Ended June 30,	
	2016	2015	2016	2015
<b>Historical</b>				
Numerator:				
Net loss	\$ (51,050)	\$ (27,133)	\$ (94,741)	\$ (51,421)
Denominator:				
Weighted average common shares outstanding basic and diluted	\$ 129,122,175	\$ 99,994,125	\$ 127,160,943	\$ 97,888,573

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

	As of June 30,	
	2016	2015
Options to purchase common stock	14,271	11,663
Outstanding warrants, convertible to common stock	3,110	
Unvested restricted stock units	322	675
Total number of potentially issuable shares	17,703	12,338

**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' response is due on August 25, 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.

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**Note 13. Subsequent Events**

On July 5, 2016, the Company entered into a Plan of Merger (the "Merger 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 Merger 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 is currently assessing the accounting impact of the merger with MiaMed.

In July 2016, as mentioned in Note 1. Description of Business, the Company sold through the ATM program, an additional 6.8 million shares of Common Stock with a net proceeds of \$39.3 million. In connection with the July share sales, the Company completed all sales under the ATM equity program.

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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 candidate, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease. SD-101, a product candidate in late-stage development, is 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 advanced 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) have been initiated and we expect to provide a U.S. regulatory update in the third 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 cell line for co-formulation with migalastat. Master cell banking has been completed and process development work has commenced. We intend to provide an update on the development of this novel ERT in Fabry disease in the second half of 2016.

We are also in Phase 3 clinical development of a novel topical cream, 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 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 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.

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. As with all LSDs, 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 amenable mutations.

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We have completed two Phase 3 global registration studies (Study 011 and Study 012) 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.

*Migalastat in Combination 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 cell line for co-formulation with migalastat. Master cell banking has been completed and process development work has commenced. We previously completed an open-label Phase 2 safety and pharmacokinetics study ( Study 013 ) that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with agalsidase beta or agalsidase alfa in males with Fabry disease. Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations, and 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 cream, 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 (SAP) 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*

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 late 2016 or early 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. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma, Inc. (Callidus).

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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 2016.

Following a positive data safety monitoring board (DSMB) 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 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**

*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 cream 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.

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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 three months ended June 30, 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.

### *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.

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During the three months ended June 30, 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 payment for this event was \$6.0 million, which was paid in common stock of the Company, par value \$0.01 per share ( Common Stock ), 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 June 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.

*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 June 30,		Six months ended June 30,	
	2016	2015	2016	2015
Third party direct project expenses				
<b>Monotherapy Studies</b>				
Migalastat (Fabry Disease Phase 3)	\$ 2,830	\$ 3,325	\$ 6,777	\$ 7,938
SD-101 (EB-Epidermolysis Bullosa Phase 3)	1,861		3,476	
<b>Combination Studies</b>				
ATB200 + AT2221 (Pompe Disease Phase 2)	799	4,390	6,589	7,980
Fabry CHART (Fabry Disease Preclinical)	37	1,023	191	1,126
Neurodegenerative Diseases (Preclinical)		2		3
Total third party direct project expenses	\$ 5,527	\$ 8,740	\$ 17,033	\$ 17,047
<b>Other project costs (1)</b>				
Personnel costs	9,149	5,964	17,568	11,534
Other costs (2)	3,605	2,530	7,105	4,766
Total other project costs	\$ 12,754	\$ 8,494	\$ 24,673	\$ 16,300
Total research and development costs	\$ 18,281	\$ 17,234	\$ 41,706	\$ 33,347

(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 June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Expected stock price volatility	81.3%	74.3%	81.2%	75.6%
Risk free interest rate	1.3%	1.7%	1.7%	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. As of June 30, 2016, Redmile beneficially owned approximately 10% of the 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 as of June 30, 2016 will settle in May 2017.

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

The loss recognized in other income/ (expense) related to foreign currency forward contract not designated as hedging instruments was \$346 thousand for the three and six months ended year ended June 30, 2016.

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**Results of Operations**

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

*Research and Development Expense.* Research and development expense was \$18.3 million during the three months ended June 30, 2016, representing an increase of \$1.1 million or 6.4% from \$17.2 million for the three months ended June 30, 2015. The increase in research and development costs was primarily due to increases in clinical research costs of \$0.3 million due to the continual progress of our programs, primarily the EB program, through the clinical development process and external program support of \$1.4 million.

*General and Administrative Expense.* General and administrative expense was \$19.3 million for the three months ended June 30, 2016, representing an increase of \$11.0 million or 132.5% from \$8.3 million for the six months ended June 30, 2015. The increase was due to personnel costs of \$4.7 million and professional fees of \$5.0 million in support of the commercial organization for the launch of Galafold.

*Changes in Fair Value of Contingent Consideration Payable.* For the three months ended June 30, 2016, we recorded expense of \$10.2 million representing an increase of \$10.1 million from the \$0.1 million of expense for the three months ended June 30, 2015. The change in the fair value resulted primarily from an increase in the Scioderm contingent consideration of \$10.9 million, partially offset by a decrease in the Callidus contingent consideration of \$0.8 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. In the second quarter of 2016, we made milestone payments of \$11.1 million.

*Loss from Extinguishment of Debt:* For the three months ended June 30, 2016, we did not recognize a loss from extinguishment of debt, as compared to \$1.0 million in the second quarter of 2015 arising from the early extinguishment of the \$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 \$8 thousand for three months ended June 30, 2016, as compared to \$26 thousand for the three months ended June 30, 2015, and was due to the change in fair value of the future minimum lease payments.

*Depreciation Expense.* Depreciation expense was \$0.8 million for the three months ended June 30, 2016, representing an increase of \$0.4 million as compared to \$0.4 million for the three months ended June 30, 2015. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2016.

*Interest Income.* Interest income was \$0.3 million for the three months ended June 30, 2016, representing an increase of \$0.1 million from \$0.2 million for the three months ended June 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 \$1.0 million for three months ended June 30, 2016, representing an increase of \$0.7 million from \$0.3 million for the three months ended June 30, 2015. Interest expense was higher due to the \$50 million notes payable secured in October 2015 and the related revised agreement in February 2016, partially offset by the early retirement of the \$15 million secured loan in June 2015.

*Other Expense.* Other expenses for the three months ended June 30, 2016 was \$2.2 million, as compared to other expenses of \$10 thousand for the three months ended June 30, 2015. The change was primarily from losses on foreign exchange transactions of \$1.9 million and \$0.4 million related to fair value changes in the forward contract.

*Tax benefit:* For the three months ended June 30, 2016, the Company recorded a discrete income tax benefit of \$0.5 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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*Six Months Ended June 30, 2016 Compared to Six Months Ended June 30, 2015*

*Research and Development Expense.* Research and development expense was \$41.7 million during the six months ended June 30, 2016, representing an increase of \$8.4 million or 25.1% from \$33.3 million for the six months ended June 30, 2015. The increase in research and development costs was primarily due to increases in clinical research costs of \$1.3 million, due to the continual progress of our programs, primarily the EB program, through the clinical development process, personnel costs of \$6.0 million and external program support of \$2.6 million.

*General and Administrative Expense.* General and administrative expense was \$35.0 million for the six months ended June 30, 2016, representing an increase of \$20.3 million or 138.1% from \$14.7 million for the six months ended June 30, 2015. The increase was due to personnel costs of \$8.9 million and professional fees of \$7.2 million. Also included within the overall increase was \$2.3 million related to pre-commercial organization costs.

*Changes in Fair Value of Contingent Consideration Payable.* For the six months ended June 30, 2016, we recorded expense of \$13.3 million representing an increase of \$12.2 million from the \$1.1 million of expense for the six months ended June 30, 2015. The change in the fair value resulted primarily from an increase in the Scioderm contingent consideration of \$13.0 million, partially offset by decrease in Callidus contingent consideration of \$0.8 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 payment of \$11.1 million.

*Loss from Extinguishment of Debt.* In the six months ended June 30, 2016, we did not recognize a loss from extinguishment of debt, as compared to \$1.0 million in the six months ended June 30, 2015 arising from the early extinguishment of the \$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 \$58 thousand for six months ended June 30, 2016 as compared to \$36 thousand for the six months ended June 30, 2015, and was due to the change in fair value of the future minimum lease payments.

*Depreciation Expense.* Depreciation expense was \$1.4 million for the six months ended June 30, 2016, representing an increase of \$0.6 million as compared to \$0.9 million for the six months ended June 30, 2015. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2016.

*Interest Income.* Interest income was \$0.6 million for the six months ended June 30, 2016, representing an increase of \$0.3 million from \$0.3 million for the six months ended June 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 \$2.0 million for six months ended June 30, 2016, representing an increase of \$1.3 million from \$0.7 million for the six months ended June 30, 2015. Interest expense was higher due to the \$50 million notes payable secured in October 2015 and the related revised agreement in February 2016, partially offset by the early retirement of the \$15 million secured loan in June 2015.

*Other Expense.* Other expenses for the six months ended June 30, 2016 was \$2.3 million, as compared to \$39 thousand for the six months ended June 30, 2015. The change was primarily from losses on foreign exchange transactions of \$2.3 million, including \$0.4 million related to fair value changes in the forward contract.

*Tax benefit.* For the six months ended June 30, 2016, the Company recorded a discrete income tax benefit of \$0.5 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*

On February 26, 2016, we entered into the Sales Agreement (the "Sales Agreement") with Cowen and Company, LLC ("Cowen") to create an at-the-market equity program under which we from time to time may offer and sell shares of our Common Stock, having an aggregate offering price of up to \$100 million through Cowen (the "ATM Facility"). Sales of the ATM Facility shares under the Sales Agreement may be made in transactions that are deemed to be "at the market offerings" as defined in Rule 415 promulgated under the Securities Act of 1933, as amended, including sales made directly on The NASDAQ Global Market, on any other existing trading market for the Common Stock or through a market maker. In addition, with our prior written approval, Cowen may also sell shares of Common Stock by any other method permitted by law, including in negotiated transactions. Cowen will act as sales agent using its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of The NASDAQ Stock Market LLC. There is no arrangement for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate up to 3.0% of the gross proceeds per share sold through it as sales agent under the Sales Agreement. Beginning in April 2016 and through June 30, 2016, we sold 8.2 million shares of Common Stock under the ATM sales agreement resulting in net proceeds of \$57.8 million, after Cowen's commission of \$1.7 million and other expenses of \$0.1 million. In July 2016, we sold an additional 6.8 million shares of Common Stock with a net proceeds of \$39.3 million after Cowen's commission. In connection with the July share sales, we 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 under 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 and the lack of any approved products to generate product sales revenue, 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, \$652.7 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 June 30, 2016, we had cash and cash equivalents and marketable securities of \$214.2 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*



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Net cash used in operations for the six months ended June 30, 2016 was \$77.7 million, due primarily to the net loss for the six months ended June 30, 2016 of \$94.7 million and the change in operating assets and liabilities of \$9.3 million. The change in operating assets and liabilities was primarily due to decrease in accounts payable and accrued expenses of \$8.2 million, partially offset by increases in prepaid assets of \$0.9 million and inventory of \$0.2 million.

Net cash used in operations for the six months ended June 30, 2015 was \$43.6 million, due primarily to the net loss for the six months ended June 30, 2015 of \$51.4 million and non-cash items such as stock based compensation of \$4.2 million, the change in fair value of the contingent consideration of \$1.1 and the loss on the extinguishment of debt of \$1.0 million. In addition there was change in operating assets and liabilities of \$0.7 million. The change in operating assets and liabilities was due to an increase in other non-current assets of \$0.5 million, partially offset by decreases in prepaid assets of \$0.6 million and decreases in accounts payable and accrued expenses of \$0.5 million.

### *Net Cash (Used in)/ Provided by Investing Activities*

Net cash used in investing activities for the six months ended June 30, 2016 was \$10.2 million and reflects \$126.9 million for the purchase of marketable securities, \$4.6 million for the acquisition of property and equipment, partially offset by \$121.3 million for the sale and redemption of marketable securities.

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Net cash provided by investing activities for the six months ended June 30, 2015 was \$31.3 million. Net cash provided by investing activities reflects \$63.1 million for the sale and redemption of marketable securities partially offset by \$30.4 million for the purchase of marketable securities and \$1.4 million for the acquisition of property and equipment.

***Net Cash Provided by Financing Activities***

Net cash provided by financing activities for the six months ended June 30, 2016 was \$82.8 million. Net cash provided by financing activities reflects \$57.8 million from issuance of Common Stock under the ATM program, \$30.0 million as proceeds from the Amended Purchase agreement and \$0.6 million from exercise of stock options, partially offset by \$5.0 million paid to Scioderm as contingent consideration and \$0.7 million from vesting of RSUs.

Net cash provided by financing activities for the six months ended June 30, 2015 was \$237.2 million. Net cash provided by financing activities reflects \$243.2 million from issuance of Common Stock, \$6.9 million from exercise of stock options and \$4.0 million from exercise of warrants, partially offset by \$15.3 million from paying off the secured loan and \$1.6 million from vesting of RSUs.

***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.

We anticipate that we will generate revenue from commercial 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 second half 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 Mt. Sinai School of Medicine ( 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 has 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 Mt. Sinai School of Medicine 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 three months ended June 30, 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 three months ended June 30, 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 three months ended June 30, 2016.

To date, we have not made any royalty payments on sales of our products.

### ***Recent Accounting Pronouncements***

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

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**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 June 30, 2016, we held \$214.2 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 June 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**

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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 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' response is due on August 25, 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**

There have been no material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015.

The Company has operations in the United Kingdom and other member countries of the European Union. On June 23, 2016, voters in the United Kingdom approved an advisory referendum to withdraw from the European Union. The specifics of how the UK will exit the EU will be the subject of negotiations for at least the next two years. This political uncertainty may further exacerbate many of the Company's risks and uncertainties. There can be no assurance that any or all of these events will not have a material adverse effect on our business operations, results of operations and financial condition.



Table of Contents**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 six months ended June 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>
May 1, 2016 to May 31, 2016	13,632	6.57		23,868
Total	13,632			23,868

There were no purchases of our Common Stock during the periods April 1, 2016 to April 30, 2016 and June 1, 2016 to June 30, 2016.

Pursuant to a restricted stock award dated April 10, 2014 between Amicus and certain employee recipients, certain employees were granted RSUs. Some of the RSUs that vested in 2015 were released in the six months ended June 30, 2016. The remainder of the RSUs will vest in July 2016, subject generally to the employee's continued employment with the Company. In order to comply with the minimum statutory federal tax withholding rate of 25%, 1.45% for Medicare plus 6.2% for Social Security where applicable, and state tax withholding of 9.9%, the employee surrendered to us a portion of the vested shares on the vesting date, representing between 36.40-42.60% of the total value of the shares then vested.

**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**

Exhibit Number	Description
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)	Management Bonus Program Summary
10.2 (5)	Amended and Restated Amicus Therapeutics, Inc. 2007 Equity Incentive Plan
10.3 (6)	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
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 six months ended June 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 June 9, 2016.
  - (5) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on June 13, 2016.
  - (6) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on July 1, 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: August 9, 2016

By:

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

Date: August 9, 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>
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