

**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 March 31, 2015

OR

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

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

Commission file number 001-33497

**Amicus Therapeutics, Inc.**

(Exact Name of Registrant as Specified in Its Charter)

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

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

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

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

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

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

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

Large accelerated filer   
Non-accelerated filer

Accelerated filer   
Smaller Reporting Company

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of April 24, 2015 was 96,421,182 shares.

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We have registered or filed applications to register certain trademarks in the United States and abroad, including AMICUS™, AMICUS THERAPEUTICS™ (and design) and CHART™ (and design).

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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 intended 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, including migalastat HCl (“migalastat”);
- 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 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 and technologies; 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, 2014 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 documents 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)

	<u>March 31, 2015</u>	<u>December 31, 2014</u>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 28,827	\$ 24,074
Investments in marketable securities	119,940	127,601
Prepaid expenses and other current assets	2,484	2,902
Total current assets	<u>151,251</u>	<u>154,577</u>
Investments in marketable securities	2,804	17,464
Property and equipment, less accumulated depreciation of \$12,028 and \$11,520 at March 31, 2015 and December 31, 2014, respectively	3,056	2,811
In-process research & development	23,000	23,000
Goodwill	11,613	11,613
Other non-current assets	892	502
<b>Total Assets</b>	<b><u>\$ 192,616</u></b>	<b><u>\$ 209,967</u></b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 15,833	\$ 16,345
Current portion of secured loan	5,189	3,840
Total current liabilities	<u>21,022</u>	<u>20,185</u>
Deferred reimbursements	36,620	36,620
Secured loan, less current portion	9,208	10,510
Contingent consideration payable	11,700	10,700
Deferred tax liability	9,186	9,186
Other non-current liability	870	588
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 125,000,000 shares authorized, 96,375,015 shares issued and outstanding at March 31, 2015 95,556,277 shares issued and outstanding at December 31, 2014	1,024	1,015
Additional paid-in capital	574,757	568,743
Accumulated other comprehensive income	(35)	(132)
Accumulated deficit	(471,736)	(447,448)
Total stockholders' equity	<u>104,010</u>	<u>122,178</u>
<b>Total Liabilities and Stockholders' Equity</b>	<b><u>\$ 192,616</u></b>	<b><u>\$ 209,967</u></b>

*See accompanying notes to consolidated financial statements*

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

	<u>Three Months Ended March 31,</u>	
	<u>2015</u>	<u>2014</u>
<b>Revenue:</b>		
Research revenue	\$ —	\$ 456
Total revenue	<u>—</u>	<u>456</u>
<b>Operating Expenses:</b>		
Research and development	\$ 16,113	\$ 9,992
General and administrative	6,427	5,176
Changes in fair value of contingent consideration payable	1,000	505

Restructuring charges	10	(8)
Depreciation	508	412
Total operating expenses	24,058	16,077
Loss from operations	(24,058)	(15,621)
Other income (expenses):		
Interest income	171	42
Interest expense	(372)	(355)
Other expense	(29)	(9)
Net loss	\$ (24,288)	\$ (15,943)
Net loss per common shares — basic and diluted	\$ (0.25)	\$ (0.25)
Weighted-average common shares outstanding — basic and diluted	95,743,416	64,353,952

See accompanying notes to consolidated financial statements

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

	Three Months Ended March 31,	
	2015	2014
Net loss	\$ (24,288)	\$ (15,943)
Other comprehensive income/ (loss):		
Unrealized gain on available-for-sale securities	97	1
Other comprehensive gain before income taxes	97	1
Provision for income taxes related to other comprehensive (loss)/ income items (a)	—	—
Other comprehensive income	97	1
Comprehensive loss	\$ (24,191)	\$ (15,942)

(a) — Taxes have not been accrued on unrealized gain on securities as the Company is in a loss position for all periods presented.

See accompanying notes to consolidated financial statements

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

	Three Months Ended March 31,	
	2015	2014
<b>Operating activities</b>		
Net loss	\$ (24,288)	\$ (15,943)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	83	59
Depreciation	508	412
Stock-based compensation	1,960	1,260
Restructuring charges	10	(8)
Non-cash changes in the fair value of contingent consideration payable	1,000	505
Changes in operating assets and liabilities:		
Receivable due from collaboration agreements	—	589
Prepaid expenses and other current assets	418	3,893
Other non-current assets	(397)	—
Accounts payable and accrued expenses	(551)	(987)
Non-current liabilities	282	—
Net cash used in operating activities	(20,975)	(10,220)
<b>Investing activities</b>		
Sale and redemption of marketable securities	40,739	11,465
Purchases of marketable securities	(18,321)	(17,227)
Purchases of property and equipment	(753)	(40)
Net cash provided by/(used in) investing activities	21,665	(5,802)
<b>Financing activities</b>		

Payments of secured loan agreement	—	(100)
Proceeds from exercise of stock options	4,063	15
Net cash provided by/(used in) financing activities	4,063	(85)
Net increase/(decrease) in cash and cash equivalents	4,753	(16,107)
Cash and cash equivalents at beginning of period	24,074	43,640
Cash and cash equivalents at end of period	\$ 28,827	\$ 27,533

#### Supplemental disclosures of cash flow information

Cash paid during the period for interest	\$ 209	\$ 208
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See accompanying notes to consolidated financial statements

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### Amicus Therapeutics, Inc.

#### Notes to Consolidated Financial Statements

#### 1. Description of Business

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

Amicus Therapeutics, Inc. (the “Company”) was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders (“LSDs”). The Company’s lead product candidate is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy (“ERT”) for Fabry disorder. The Company’s development programs also include next-generation ERTs for LSDs, including Fabry disorder, Pompe disorder and Mucopolysaccharoidosis Type I (“MPS I”). The Company’s activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development.

We have completed two Phase 3 global registration studies of migalastat monotherapy.

During the first quarter of 2015, we met with regulatory authorities in Europe and the U.S. to discuss the approval pathways for migalastat as a monotherapy for Fabry patients who have amenable mutations. In Europe, we plan to submit a marketing authorization application (“MAA”) to the European Medicines Agency (“EMA”) in the second quarter of 2015. In the U.S., we plan to submit a new drug application (“NDA”) for accelerated approval (Subpart H) with the U.S. Food and Drug Administration (“FDA”) in the second half of 2015.

In November 2014, the Company issued a total of 15.9 million shares through a public offering at a price of \$6.50 per share, with net proceeds of \$97.2 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disorder, the continued clinical development of its product candidates and for other general corporate purposes.

The Company had an accumulated deficit of approximately \$471.7 million at March 31, 2015 and anticipates incurring losses through the fiscal year ending December 31, 2015 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund 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 (“IPO”) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. 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 2016.

#### 2. Summary of Significant Accounting Policies

##### *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, 2014. 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, 2014.

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##### *Significant Accounting Policies*

There have been no material changes to the Company’s significant accounting policies during the three months ended March 31, 2015, 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, 2014. However, the following accounting policies are the most critical in fully understanding and evaluating the Company’s financial condition and results of operations.

## ***Revenue Recognition***

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.

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

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

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

- a payment for an identifiable benefit; and
- the identifiable benefit is separable from the existing relationship between the Company and its customer; and
- 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.

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### ***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 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.

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

In April 2015, the FASB issued Accounting Standards Update (“ASU”) 2015-03, *Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs*. The amendments in ASU 2015-03 are intended to simplify the presentation of debt issuance costs. These amendments require that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. The recognition and measurement guidance for debt issuance costs are not affected by the amendments in this ASU. The ASU is effective for financial statements issued for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. We are currently assessing the impact that this standard will have on our consolidated financial statements.

In November 2014, the FASB issued ASU 2014-17, *Business Combinations (Topic 805): Pushdown Accounting*. The amendments in ASU 2014-17 provide an acquired entity with an option to apply pushdown accounting in its separate financial statements upon occurrence of an event in which an acquirer obtains control of the acquired entity. The ASU is effective on November 18, 2014. After the effective date, an acquired entity can make an election to apply the guidance to future change-in-control events or to its most recent change-in-control event. However, if the financial statements for the period in which the most recent change-in-control event occurred already have been issued or made available to be issued, the application of this guidance would be a change in accounting principle. We are currently assessing the impact that this standard will have on our consolidated financial statements.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements-Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern*, which defines management’s responsibility to assess an entity’s ability to continue as a going concern, and to provide related footnote disclosures if there is substantial doubt about its ability to continue as a going concern. The pronouncement is effective for annual reporting periods ending after December 15, 2016 with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on our consolidated financial statements.

In May 2014, FASB issued ASU 2014-09, *Revenue From Contracts With Customers*, that outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The ASU is based on the principle that an entity should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to fulfill a contract. Entities have the option of using either a full retrospective or a modified retrospective approach for the adoption of the new standard. The ASU becomes effective for us at the beginning of our 2017 fiscal year; early adoption is not permitted. We are currently assessing the impact that this standard will have on its consolidated financial statements.

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

As of March 31 2015, the Company held \$28.8 million in cash and cash equivalents and \$122.7 million of available-for-sale 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/ (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.

Consistent with the Company’s investment policy, the Company does not use derivative financial instruments in its investment portfolio. 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.

Cash and available-for-sale securities consisted of the following as of March 31, 2015 and December 31, 2014 (in thousands):

	As of March 31, 2015			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	\$ 28,827	\$ —	\$ —	\$ 28,827
Corporate debt securities, current portion	107,135	3	(47)	107,091
Corporate debt securities, non-current portion	2,808	—	(4)	2,804
Commercial paper	12,486	13	—	12,499
Certificate of deposit	350	—	—	350
	<u>\$ 151,606</u>	<u>\$ 16</u>	<u>\$ (51)</u>	<u>\$ 151,571</u>
Included in cash and cash equivalents	\$ 28,827	\$ —	\$ —	\$ 28,827
Included in marketable securities	122,779	16	(51)	122,744
Total cash and marketable securities	<u>\$ 151,606</u>	<u>\$ 16</u>	<u>\$ (51)</u>	<u>\$ 151,571</u>

  

	As of December 31, 2014			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	\$ 24,074	\$ —	\$ —	\$ 24,074
Corporate debt securities, current portion	115,862	—	(110)	115,752
Corporate debt securities, non-current portion	17,508	—	(44)	17,464
Commercial paper	11,477	22	—	11,499
Certificate of deposit	350	—	—	350
	<u>\$ 169,271</u>	<u>\$ 22</u>	<u>\$ (154)</u>	<u>\$ 169,139</u>
Included in cash and cash equivalents	\$ 24,074	\$ —	\$ —	\$ 24,074
Included in marketable securities	145,197	22	(154)	145,065
Total cash and marketable securities	<u>\$ 169,271</u>	<u>\$ 22</u>	<u>\$ (154)</u>	<u>\$ 169,139</u>



Unrealized gains and losses are reported as a component of other comprehensive income/ (loss) in the statements of comprehensive loss. For the three months ended March 31, 2015, unrealized holding gain of \$97 thousand and for the year ended December 31, 2014, unrealized holding loss of \$132 thousand, included in the statement of comprehensive loss.

For the three months ended March 31, 2015 and the year ended December 31, 2014, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

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Unrealized loss positions in the available for sale securities as of March 31, 2015 and December 31, 2014 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was \$86.2 million and \$129.2 million as of March 31, 2015 and December 31, 2014, 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. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the three months ended March 31, 2015 and March 31, 2014 were as follows (in thousands):

	Three Months Ended March 31,	
	2015	2014
Balance, beginning	\$ (132)	\$ 1
Current period changes in fair value, (a)	97	1
Reclassification of earnings, (a)	—	—
Balance, ending	<u>\$ (35)</u>	<u>\$ 2</u>

(a) — Taxes have not been accrued on the unrealized gain on securities as the Company is in a loss position for all periods presented.

**Note 4. 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 and its lead ERT is ATB200 for Pompe disorder in late preclinical development. The acquisition of the Callidus assets and technology compliments Amicus' CHART™ platform for the development of next generation ERTs.

In consideration for the merger, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value \$0.01 per share, to the former stockholders of Callidus. As of March 31, 2015, approximately 25 thousand shares remain issuable to former Callidus shareholders. In addition, the Company 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. The Company may, at its election, satisfy certain milestone payments identified in the Merger Agreement aggregating \$40 million in shares of its 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 Market for the ten (10) 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 Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the Merger Agreement, the rules of The NASDAQ Global Market, or otherwise, will be paid in cash.

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. Key assumptions included a discount rate of 11.0% and various probability factors. As of March 31, 2015, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes. (see "— Note 8. 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 \$11.7 million at March 31, 2015, resulting in an increase in the contingent consideration payable and related expense of \$1.0 million for the three months ended March 31, 2015. The expense is recorded as part of operating expense in the Consolidated Statement of Operations.

For further information, see — Note 5 Goodwill & — Note 6 Intangible Assets.

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**Note 5. Goodwill**

In connection with the acquisition of Callidus as discussed in "—Note 4. Acquisition of Callidus Biopharma, Inc.", the Company recognized goodwill of \$11.6 million. Goodwill is 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. During the 2014 impairment assessment, it was determined that the goodwill had not been impaired and there were no changes to the goodwill balance in 2014. For the three months ended March 31, 2015, there were no indicators of impairment and the goodwill balance remained at \$11.6 million.

**Note 6. Intangible Assets**



In connection with the acquisition of Callidus as discussed in “—Note 4. Acquisition of Callidus Biopharma, Inc.”, the Company recognized In Process Research & Development (“IPR&D”) of \$23.0 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 on October 1 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. During the 2014 impairment assessment, it was determined that the IPR&D had not been impaired and there was no change in the IPR&D balance in 2014. For the three months ended March 31, 2015, there were no indicators of impairment and the IPR&D balance remained at \$23.0 million.

## Note 7. Stockholders’ Equity

### Common Stock and Warrants

As of March 31, 2015, the Company was authorized to issue 125 million shares of common stock. Dividends on common stock will be paid when, and if declared by the board of directors. Each holder of common stock is entitled to vote on all matters that are appropriate for stockholder voting and is entitled to one vote for each share held.

In November 2014, we sold a total of 15.9 million shares of our common stock, par value \$0.01 per share, at a public offering price of \$6.50 per share. The aggregate offering proceeds were approximately \$97.2 million. We expect to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disorder, the continued clinical development of its product candidates and for other general corporate purposes.

In July 2014, the Company completed a \$40 million at the market (“ATM”) equity offering under which the Company sold shares of its common stock, par value \$0.01 per shares with Cowen and Company LLC as sales agent. Under the ATM equity program the Company sold 14.3 million shares of common stock resulting in net proceeds of \$38.6 million.

The warrants issued in connection with the November 2013 securities and purchase agreement (“SPA”) are classified as equity. As part of the SPA, a total of 7.5 million common shares and 1.6 million warrants were issued at \$2.00 per share, for total cash received of \$15 million. The warrants are included in stockholder’s equity and were initially measured at fair value of \$1.0 million using the Black Scholes valuation model.

### Nonqualified Cash Plan

In July 2014, the Board of Directors approved the Company’s Deferral Plan, (the “Deferral Plan”) which 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 March 31, 2015 were approximately \$0.4 million, as compared to \$0.1 million on December 31, 2014 and are included in other long term liabilities. As of December 31, 2014, the amounts deferred under the Deferral Plan had not been invested and the investments were subsequently made in the three months ended March 31, 2015. Deferral Plan assets as of March 31, 2015 were \$0.4 million are classified as trading securities and recorded at fair value with changes in the investments’ fair value recognized in the period they occur. During the three months ended March 31, 2015, income from the investments was under \$1 thousand and unrealized gain/loss was under \$1 thousand.

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### Equity Incentive Plan

#### Stock Option Grants

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

	Three Months ended March 31,	
	2015	2014
Expected stock price volatility	77.1%	81.4%
Risk free interest rate	1.7%	2.0%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00

A summary of the Company’s stock options for the three months ended March 31, 2015 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, 2014	10,020.7	\$ 5.02		
Options granted	1,415.7	\$ 9.07		
Options exercised	(818.7)	\$ 5.02		
Options forfeited	(15.0)	\$ 2.80		
Balance at March 31, 2015	10,602.7	\$ 5.56	7.3 years	\$ 57.6
Vested and unvested expected to vest March 31, 2015	9,842.5	\$ 5.58	7.2 years	\$ 53.4
Exercisable at March 31, 2015	5,261.0	\$ 6.30	5.7 years	\$ 25.3

As of March 31, 2015, the total unrecognized compensation cost related to non-vested stock options granted was \$13.4 million and is expected to be recognized over a weighted average period of 3.0 years.

#### Restricted Stock Units

A summary of non-vested Restricted Stock Units (“RSU”) activity under the Plan for the three months ended March 31, 2015 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, 2014	955	\$ 2.28		
Granted	—	\$ —		
Vested	—	\$ —		
Forfeited	—	\$ —		
Non-vested units as of March 31, 2015	<u>955</u>	\$ 2.28	0.49	\$ 8.2

For the three months ended March 31, 2015, there were no RSUs that vested and all non-vested units are expected to vest over their normal term. As of March 31, 2015, there was \$0.7 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 0.49 years.

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#### 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 March 31,	
	2015	2014
Equity compensation expense recognized in:		
Research and development expense	\$ 947	\$ 550
General and administrative expense	1,013	710
Total equity compensation expense	<u>\$ 1,960</u>	<u>\$ 1,260</u>

#### Note 8. 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.

#### 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 three March 31, 2015. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 2015.

#### Secured Debt

As disclosed in “—Note 9. Short Term Borrowings and Long Term Debt”, the Company has a loan and security agreement with MidCap Financial, Oxford Finance and Silicon Valley Bank (“Term Loan”). The carrying amount of the Company’s borrowings approximates fair value at March 31, 2015. The Company’s secured debt is classified as Level 2 and the fair value is estimated using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals.

In connection with the Term Loan, as disclosed in “—Note 9. Short Term Borrowings and Long Term Debt”, the Company recorded a contingent liability of approximately \$0.3 million representing the fair value of a contingent payment of up to \$0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. This is effective 5 years from the closing of the Term Loan. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and hence classified as Level 3.

#### Contingent Consideration Payable

The contingent consideration payable resulted from acquisition of Callidus, as discussed in “—Note 4. Acquisition of Callidus Biopharma, Inc.” Our 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. Some of the more significant assumptions used in the valuation include (i) ATB200 clinical forecasts (ii) the probability and timing related to the achievement of certain developmental

milestones and (iii) and the discount rate of 11.0% which is a measure of the credit risk associated with settling the liability. The probability of achievement of clinical milestones ranged from 24% to 75% with milestone payment outcomes ranging from \$0 to \$81 million. The valuation is

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performed quarterly. Gains and losses are included in the statement of operations. There is no assurance that any of the conditions for the milestone payments will be met.

The contingent consideration payable has been classified as a Level 3 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.

**Deferred Compensation Plan- Investment and Liability**

As disclosed in “—Note 7. Stockholders’ Equity”, the Deferral Plan provides certain key employees and members of the Board of Directors with an opportunity to defer the receipt of such Participant’s base salary, bonus and director’s fees, as applicable. Deferral Plan assets as of March 31, 2015 were \$0.4 million are classified as trading securities and recorded at fair value with changes in the investments’ fair value recognized in the period they occur. During the three months ended March 31, 2015, the unrealized gain/loss was under \$1 thousand. 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.

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 March 31, 2015, are identified in the following table (in thousands):

	Level 1	Level 2	Total	
<b>Assets:</b>				
Cash/ money market funds	\$ 28,827	\$ —	\$ 28,827	
Corporate debt securities	—	109,895	109,895	
Commercial paper	—	12,499	12,499	
Certificate of deposit	—	350	350	
Deferred Compensation Plan Assets	—	398	398	
	<u>\$ 28,827</u>	<u>\$ 123,142</u>	<u>\$ 151,969</u>	
<b>Liabilities:</b>				
Contingent success fee payable	—	—	370	370
Contingent consideration payable	—	—	11,700	11,700
Deferred Compensation Plan Liability	—	406	—	406
	<u>\$ —</u>	<u>\$ 406</u>	<u>\$ 12,070</u>	<u>\$ 12,476</u>

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, 2014, are identified in the following table (in thousands):

	Level 1	Level 2	Total	
<b>Assets:</b>				
Cash/ money market funds	\$ 24,074	\$ —	\$ 24,074	
Corporate debt securities	—	133,216	133,216	
Commercial paper	—	11,499	11,499	
Certificate of deposit	—	350	350	
Deferred Compensation Plan Assets	—	—	—	
	<u>\$ 24,074</u>	<u>\$ 145,065</u>	<u>\$ 169,139</u>	
<b>Liabilities:</b>				
Contingent success fee payable	—	—	341	341
Contingent consideration payable	—	—	10,700	10,700
Deferred Compensation Plan Liability	—	124	—	124
	<u>\$ —</u>	<u>\$ 124</u>	<u>\$ 11,041</u>	<u>\$ 11,165</u>

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**Note 9. Short-Term Borrowings and Long-Term Debt**

In December 2013, the Company entered into a credit and security agreement with a lending syndicate consisting of MidCap Funding III, LLC, Oxford Finance LLC, and Silicon Valley Bank. The Company drew \$15 million of the aggregate principal amount which bears interest at a rate per annum fixed at 8.5%. The Company made interest-only payments on the Term Loan beginning January 1, 2014 and will continue through April 1, 2015, after which the Company will repay the aggregate principal outstanding balance of the Term Loan in 33 equal monthly installments of principal, plus accrued interest at the applicable rate. The Term Loan matures on December 27, 2017. At March 31, 2015 the total principal amount due under the Term Loan was \$15 million.

In connection with the Term Loan, the Company recorded a debt discount of \$0.8 million at December 31, 2013 which consists of payments to be made and a contingent payable to the lenders. These payments include a debt facility fee of \$0.1 million which was paid on the date of the First Tranche, \$0.4 million exit fee that will be payable upon repayment of the term loan and \$0.4 million representing the fair value of a contingent payment of up to \$0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. This is effective 5 years from the closing of the Term Loan. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and is shown as a current liability on the Company's consolidated balance sheet.

**Note 10. Collaborative Agreements**

*GSK*

In November 2013, Amicus entered into the Revised Agreement with GlaxoSmithKline ("GSK"), pursuant to which Amicus has obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disorder. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from Amicus 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.

Under the terms of the Revised Agreement, GSK will no longer jointly fund development costs for all formulations of migalastat.

*Biogen*

In September 2013, the Company entered into a license and collaboration agreement (the "Biogen Agreement") with Biogen Idec ("Biogen") to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disorder. Under terms of the agreement, the Company and Biogen collaborated in the discovery of a new class of small molecules that target the GCase enzyme, for further development and commercialization by Biogen. Biogen was responsible for funding all discovery, development, and commercialization activities. In addition the Company was reimbursed for all full-time employees working on the project as part of a cost sharing arrangement. The Company was also eligible to receive development and regulatory milestones, as well as modest royalties in global net sales.

In accordance with the revenue recognition guidance related to reimbursement of research and development expenses, the Company identified all deliverables at the inception of the agreement. As the Company has not commenced its planned principal operations (i.e. selling commercial products) the Company is only performing development of its compounds, and therefore, development activities are part of the Company's ongoing central operations. Additionally, the Company has the following accounting policies:

- Research and development expenses related to a collaboration agreement will be recorded on a gross basis in the income statement and not presented net of any reimbursement received from a collaboration agreement; and
- The reimbursement of research and development expenses from a collaborator will be recognized in the income statement as "Research Revenue" for the period in which the research activity occurred.

For the three months ended March 31, 2014, the Company recognized \$0.5 million in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

In September 2014, the Company and Biogen concluded their research collaboration. The Company's most advanced Parkinson's candidate is AT3375, which was developed outside the collaboration and is wholly-owned by the Company.

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**Note 11. 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 three months ended March 31, 2015 (in thousands):

	Balance as of December 31, 2014	Charges	Cash Payments	Adjustments	Balance as of March 31, 2015
Facilities consolidation	\$ 283	\$ —	\$ (59)	\$ 10	\$ 234

**Note 12. Subsequent Events**

The Company evaluated events that occurred subsequent to March 31, 2015 and there were no material recognized or non-recognized subsequent events during this period.

**Note 13. Basic and Diluted Net Loss Attributable to Common Stockholders 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 attributable to common stockholders per common share:

(In thousands, except per share amounts)	Three Months Ended March 31,	
	2015	2014

## Historical

Numerator:

Net loss attributable to common stockholders	\$ (24,288)	\$ (15,943)
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## Denominator

Weighted average common shares outstanding — basic and diluted	95,743,416	64,353,952
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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 March 31,	
	2015	2014
Options to purchase common stock	10,603	9,660
Outstanding warrants, convertible to common stock	1,600	1,600
Unvested restricted stock units	955	—
Total number of potentially issuable shares	13,158	11,260

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

### Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders ("LSDs"). Our lead product candidate is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy ("ERT") for Fabry disorder. Our development programs also include next-generation ERTs for LSDs, including Fabry disorder, Pompe disorder and Mucopolysaccharidosis Type I ("MPS I"). We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of developing therapies for rare and orphan diseases.

### Program Status

Our personalized medicine approach consists of an oral small molecule pharmacological chaperone monotherapy that is designed to bind to and stabilize a patient's own endogenous target protein. Patients with "amenable mutations" may respond based on their genetics. Our Chaperone-Advanced Replacement Therapy, or CHART™, platform combines chaperones with ERTs independent of a patient's own genetics. In each CHART program, a unique pharmacological chaperone is designed to bind to a specific therapeutic (exogenous) enzyme, stabilizing the enzyme in its properly folded and active form. This may allow for enhanced tissue uptake, greater lysosomal activity, more reduction of substrate, and the potential for lower immunogenicity.

Our Fabry franchise strategy is to develop the pharmacological chaperone migalastat HCl ("migalastat") for all patients with Fabry disorder - as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients.

#### *Migalastat for Fabry Disorder as a Monotherapy*

We have completed two Phase 3 global registration studies (Study 011 and Study 012) of migalastat monotherapy and plan to submit marketing applications in the United State and Europe in 2015. We have reported Phase 3 data in both treatment naïve patients ("Study 011" or "FACETS") and enzyme replacement therapy ("ERT") switch patients ("Study 012" or "ATTRACT"). Positive results from these studies have shown that treatment with migalastat has resulted in reductions in disorder substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations in a validated assay ("GLP HEK assay").

Study 011 was a 24-month study of Fabry disorder patients naïve to or not receiving ERT, which investigated the safety and efficacy of oral migalastat. The study consisted of a 6-month double-blind, placebo-controlled period, a 6-month open-label period, and a 12-month open-label extension phase. Subjects completing Study 011 were eligible to continue treatment with migalastat in a long-term open-label extension ("Study 041"). 67 subjects (24 male) were enrolled. All subjects enrolled in Study 011 had amenable mutations in the clinical trial human embryonic kidney ("HEK") cell-based in vitro assay that was available at study initiation ("clinical trial assay"). Following the completion of enrollment, a GLP-validated HEK assay was developed with a third party to measure the criteria for amenability with more quality control and rigor ("GLP HEK assay"). Approximately 10% of mutations in the HEK database switched categorization between "amenable" and "non-amenable" when moving from the clinical trial assay to the GLP HEK assay. Therefore, there were changes in categorization from amenable to non-amenable in 17 of the 67 patients enrolled in Study 011.

Study 011 was designed to measure the reduction of the disorder substrate (Globotriaosylceramide, or "GL-3") in the interstitial capillaries of the kidney following treatment with oral migalastat (150 mg every other day). The study also measured clinical outcomes, including renal function, as secondary endpoints.

As previously reported, patients on migalastat experienced greater reductions in GL-3 as compared to placebo during the initial 6-month period; however, this difference was not statistically significant under the original analysis of the primary endpoint (responder analysis with a 50% reduction threshold at month 6). The variability and low levels of GL-3 at baseline contributed to a higher-than-anticipated placebo response at month 6.

Following the unblinding of the 6-month data, and while still blinded to the 12-month data, we reported the mean change in GL-3 from the baseline to month 6 as a post-hoc analysis in the subgroup of patients with GLP HEK-amenable mutations. This analysis showed a statistically significant reduction in GL-3 in the migalastat group compared to placebo. The mean change in GL-3 was identified as a more appropriate way to control for the variability in GL-3 levels in Study 011 and to measure the biological effect of migalastat.

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Results from this subgroup analysis further support use of the GLP HEK assay in predicting responsiveness to migalastat. Following a Type C Meeting with the U.S. Food and Drug Administration (“FDA”), we revised the Statistical Analysis Plan to pre-specify the primary analysis at month 12 as mean change in interstitial capillaries GL-3 in patients with GLP HEK amenable mutations.

Throughout 2014 and in early 2015, we announced positive 12- and 24-month data from Study 011 and longer-term data from Study 041 in patients with amenable mutations who were naïve to ERT. Top-line data were announced in April 2014 and presented to the scientific community at the American Society of Human Genetics (“ASHG”) in October 2014 and WORLDSymposium™ in February 2015. Highlights were as follows:

- Subjects who switched from placebo to migalastat after month 6 demonstrated a statistically significant reduction in disorder substrate, or kidney interstitial capillary GL-3, at month 12 ( $p=0.013$ ), and a statistically significant reduction of disorder substrate in another important biomarker of disorder, plasma lyso-Gb3. Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.
- Kidney function, as measured by estimated glomerular filtration rate (“eGFR”) and iohexol measured GFR (“mGFR”), remained stable following 18-24 months of treatment with migalastat in Study 011. Kidney function, as measured by eGFR, continued to remain stable in patients receiving migalastat in Study 011 for at least 18 months and continuing migalastat treatment in Study 041 for an average of 32 months. mGFR was not collected in Study 041.
- Reduction in cardiac mass, as measured by left ventricular mass index (“LVMI”), was statistically significant following treatment with migalastat for up to 36 months (average of 22 months) in patients in Study 011 and 041.
- There was a significant decrease in diarrhea (unadjusted  $p=0.03$ ) in patients treated with migalastat versus placebo during the 6-month double-blind phase (Stage 1). After 18-24 months of treatment with migalastat, significant improvements in diarrhea and indigestion were observed in addition to favorable trends in reflux and constipation. Gastrointestinal symptoms were assessed using the Gastrointestinal Symptoms Rating Scale (“GSRS”), a validated instrument
- Migalastat was generally safe and well-tolerated

Study 012, our second Phase 3 registration study, was a randomized, open-label 18-month study that investigated the safety and efficacy of oral migalastat (150 mg, every other day) compared to standard-of-care infused ERTs (agalsidase beta and agalsidase alfa). The study also included a 12-month open-label migalastat extension phase. The study enrolled a total of 60 patients (males and females) with Fabry disorder and genetic mutations identified as amenable to migalastat monotherapy in the clinical trial assay. Subjects were randomized 1.5:1 to switch to migalastat or remain on ERT. All subjects had been receiving ERT infusions for a minimum of 12 months (at least 3 months at the labeled dose) prior to entering the study. Based on the GLP HEK assay, there were changes in categorization from amenable to non-amenable in 4 of the 60 patients enrolled in Study 012.

Taking into account scientific advice from European regulatory authorities, the pre-specified co-primary outcome measures of efficacy in Study 012 are the descriptive assessments of comparability of the mean annualized change in mGFR and eGFR for migalastat and ERT. Both mGFR and eGFR are considered important measures of renal function. Success on mGFR and eGFR was prescribed to be measured in two ways: 1) a 50% overlap in the confidence intervals between the migalastat and ERT treatment groups; and 2) whether the mean annualized changes for patients receiving migalastat are within 2.2 mL/min/1.73 m<sup>2</sup>/yr of patients receiving ERT. We pre-specified that these renal function outcomes would be analyzed in patients with GLP HEK amenable mutations.

In August 2014, we announced positive 18-month data from the Study 012. Data from Study 012 were also presented to the scientific community at the American Society of Nephrology (“ASN”) in November 2014 and WORLDSymposium in February 2015. Highlights were as follows:

- Migalastat had a comparable effect to ERT on patients’ kidney function as measured by the change in eGFR and mGFR from baseline to month 18.
- Levels of plasma lyso-Gb3, an important biomarker of disorder, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.
- There was a statistically significant decrease in LVMI from baseline to month 18 in patients who switched from ERT to migalastat
- Measures of pain and quality of life from the Brief Pain Inventory (“BPI”) and Short Form 36 (“SF36”) remained stable when patients switched from ERT to migalastat.
- Migalastat was generally safe and well-tolerated.

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During the first quarter of 2015, we met with regulatory authorities in Europe and the U.S. to discuss the approval pathways for migalastat as a monotherapy for Fabry patients who have amenable mutations. In Europe, we plan to submit a marketing authorization application (“MAA”) to the European Medicines Agency (“EMA”) in the second quarter of 2015. In the U.S., we plan to submit a new drug application (“NDA”) for accelerated approval (Subpart H) with the U.S. Food and Drug Administration (“FDA”) in the second half of 2015.

#### *Migalastat Combination Programs for Fabry Disorder*

In support of our Fabry Franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations, we plan to conduct a longer-term Phase 2 Fabry co-administration study in 2015. In parallel, we are internally developing our own Fabry cell line for co-formulation with migalastat as a next-generation ERT for Fabry disorder. 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 disorder. Unlike Study 011 and Study 012, patients in Study 013 were not required to have alpha-Gal A mutations amenable to chaperone therapy because, when co-administered with ERT, migalastat is designed to bind to and stabilize the exogenous enzyme in the circulation in any patient receiving ERT. Each patient received their current dose and regimen of ERT at one infusion. A single oral dose of migalastat (150 mg or 450 mg) was co-administered two hours prior to the next infusion of the same ERT at the same dose and regimen. Preliminary results from Study 013 showed increased levels of active alpha-Gal A enzyme levels in plasma and skin following co-administration compared to ERT alone.



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

In preclinical studies, ATB200 demonstrated greater tissue enzyme levels and further substrate reduction compared to the current approved ERT for Pompe disorder (alglucosidase alfa), which were further improved with the addition of a chaperone. Clinical studies of pharmacological chaperones in combination with currently marketed ERTs have established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability. In 2013, we completed a Phase 2 safety and pharmacokinetics study (“Study 010”) that investigated single ascending oral doses of a pharmacological chaperone co-administered with alglucosidase alfa marketed by Genzyme, in patients with Pompe disorder. Each patient received one infusion of ERT alone, and then a single oral dose of the pharmacological chaperone just prior to the next ERT infusion. Results from this study showed an increase in GAA enzyme activity in plasma and muscle when co-administered compared to ERT alone.

Taken together, these clinical results support further development of ATB200 in combination with a pharmacological chaperone as a next-generation Pompe ERT. The initiation of a Phase 1/2 clinical study is expected in the second half of 2015.

#### *Collaboration with Biogen*

In September 2013, we entered into a collaboration agreement with Biogen Idec (“Biogen”) to discover, develop and commercialize novel small molecules that target the glucocerebrosidase (“GCase”) enzyme for the treatment of Parkinson’s disorder. In September 2014, we concluded our research collaboration with Biogen. Our most advanced Parkinson’s candidate is AT3375, which was developed outside the collaboration and is wholly-owned by us.

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### *Other Potential Alliances and Collaborations*

We continually evaluate other potential collaborations and business development opportunities that would bolster our ability to develop therapies for rare and orphan diseases including licensing agreements and acquisitions of businesses and assets. We believe such opportunities may be important to the advancement of our current product candidate pipeline, the expansion of the development of our current technology, gaining access to new technologies and in our transformation to a commercial biotechnology company.

### **Acquisition of Callidus Biopharma, Inc.**

In November 2013, we entered into a merger agreement (the “Merger Agreement”) with Callidus Biopharma, Inc. (“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.

### **Critical Accounting Policies and Significant Judgments and Estimates**

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 March 31, 2015 to the items that we disclosed as our significant accounting policies and estimates described in Note 2 to the Company’s financial statements as contained in the Company’s Annual Report on Form 10-K for the year ended December 31, 2014. 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.

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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.

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 March 31,	
	2015	2014
Third party direct project expenses		
Monotherapy Studies		
Migalastat (Fabry Disorder — Phase 3)	\$ 4,613	\$ 2,914
Combination (CHART) Studies		
ATB200 + chaperone (Pompe Disorder - Preclinical)	3,590	976
Migalastat + chaperone (Fabry Disorder — Preclinical)	103	173
Neurodegenerative Diseases (Preclinical)	1	49
Total third party direct project expenses	8,307	4,112
Other project costs (1)		
Personnel costs	5,570	4,294
Other costs (2)	2,236	1,586
Total other project costs	7,806	5,880
Total research and development costs	\$ 16,113	\$ 9,992

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

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*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:

	Three Months Ended March 31,	
	2015	2014
Expected stock price volatility	77.1%	81.4%
Risk free interest rate	1.7%	2.0%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00

*Restricted Stock Units*

In April 2014, the Compensation Committee made awards of restricted stock units (“RSUs”) to certain employees of the Company. The RSUs were awarded under the Plan and are generally subject to graded vesting of 50% of the RSUs on the 13<sup>th</sup> month anniversary of the grant date and the remaining 50% of the RSUs on the 20<sup>th</sup> month anniversary of the grant date, in each case, 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.

In April 2014, our Board of Director approved the Company's Restricted Stock Unit Deferral Plan (the "Deferred Compensation Plan"), which provides selected employees with an opportunity to defer receipt of RSUs until the first to occur of termination of the employee's employment or a date selected by the employee. Any RSUs deferred under the Deferred Compensation Plan would be fully vested once the original vesting conditions of the RSUs were satisfied.

#### *Warrants*

The warrants issued in connection with our November 2013 securities and purchase agreement ("SPA") are classified as equity. As part of the SPA, a total of 7.5 million common shares and 1.6 million warrants were issued at \$2.00 per share, for total cash received of \$15 million. The warrants are included in stockholder's equity and were initially measured at fair value of \$1.0 million using the Black Scholes valuation model.

#### *Nonqualified Cash Deferral Plan*

In July 2014, our Board of Directors approved the Cash Deferral Plan (the "Deferral Plan"), which 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 "Code").

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

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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.

### **Results of Operations**

#### ***Three Months Ended March 31, 2015 Compared to Three Months Ended March 31, 2014***

**Revenue.** In September 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. This collaboration was ended in September 2014. For the three months ended March 31, 2014, we recognized \$0.5 million as Research Revenue for reimbursed research and development costs.

**Research and Development Expense.** Research and development expense was \$16.1 million during the three months ended March 31, 2015, representing an increase of \$6.1 million or 61.0% from \$10.0 million for the three months ended March 31, 2014. The increase in research and development costs was due primarily to increases in contract research and manufacturing, as well as increase in personnel costs of \$1.3 million. Contract research increased by \$2.3 million and contract manufacturing by \$2.0 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the ATB200 + chaperone program and the migalastat program, partially offset by decreases in the migalastat + chaperone program. The migalastat program also saw increased spending due to the revised agreement where we were responsible for 100% of the program costs in 2015 as compared to 40% for the three months ended March 31, 2014.

**General and Administrative Expense.** General and administrative expense was \$6.4 million the three months ended March 31, 2015, representing an increase of \$1.2 million or 23.1% from \$5.2 million for the three months ended March 31, 2014. The increase was primarily due to consulting fees of \$0.8 million, personnel costs of \$0.4 million and recruitment of \$0.2 million. These increases were partially offset by decreases in legal expenses of \$0.2 million.

**Changes in Fair Value of Contingent Consideration Payable.** For three months ended March 31, 2015, we recorded expense of \$1.0 million representing an increase of \$0.5 million or 100% from \$0.5 million for the three months ended March 31, 2014. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates.

**Restructuring Charges.** Adjustments to the restructuring liability were \$10 thousand for three months ended March 31, 2015 as compared to \$8 thousand for the three months ended March 31, 2014 and were due to the change in fair value of future minimum lease payments.

**Depreciation.** Depreciation and amortization expense was \$0.5 million for the three months ended March 31, 2015, representing an increase of \$0.1 million or 25.0% as compared to \$0.4 million for the three months ended March 31, 2014. The change was due to an increase in the amount of property, plant and equipment.

**Interest Income.** Interest income was \$171 thousand for the three months ended March 31, 2015, representing an increase of \$129 thousand or 297.7% from \$42 thousand for the three months ended March 31, 2014. The increase in interest income was due to the overall higher average cash and investment balances as a result of 2014 public offering, 2014 ATM sales and cash received from option exercises in 2014 and 2015.

**Interest Expense.** Interest expense was approximately \$0.4 million for the three months ended March 31, 2015 and March 31, 2014. Interest expense was incurred on the \$15 million loan secured in December 2013.

**Other Income/Expense.** Other income/expenses for the three months ended March 31, 2015 included charges of \$29 thousand for increase in the fair value of the success fee payable, as compared to \$9 thousand for the three months ended March 31, 2014. The success fee was related to the \$15 million secured loan in 2013.

## Liquidity and Capital Resources

### *Source of Liquidity*

In November 2014, we sold a total of 15.9 million shares of our common stock at a public offering price of \$6.50 per share. The offering generated gross proceeds of \$103.5 million. After deducting the underwriting fee of \$6.2 million and other offering expenses of \$0.1 million, which included legal fees, the net proceeds of the offering were approximately \$97.2 million. We expect to use the net proceeds of the offering for investment in the global commercialization infrastructure for migalastat monotherapy for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In July 2014, the Company completed a \$40 million at the market (“ATM”) equity offering under which the Company sold shares of its common stock, par value \$0.01 per shares with Cowen and Company LLC as sales agent. Under the ATM equity program the Company sold 14.3 million shares of common stock resulting in net proceeds of \$38.6 million.

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, \$317.6 million of gross proceeds from our stock offerings, \$130.0 million from investments by collaborators and non-refundable license fees from those collaborations.

In December 2013, we entered into a credit and security agreement with a lending syndicate which provided an aggregate of \$25 million credit available. We drew \$15 million of the aggregate principal amount in December 2013 and did not draw the additional \$10 million that was available through the end of the fourth quarter of 2014.

As of March 31, 2015, we had cash and cash equivalents and marketable securities of \$151.6 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

### *Net Cash Used in Operating Activities*

Net cash used in operations for the three months ended March 31, 2015 was \$21.0 million, due primarily to the net loss for the three months ended March 31, 2015 of \$24.3 million and the change in operating assets and liabilities of \$0.2 million. The change in operating assets and liabilities consisted of a decrease of \$0.4 million in prepaid assets primarily related to decrease in interest receivable of \$0.3 million and a decrease in accounts payable and accrued expenses of \$0.6 million related to program expenses.

Net cash used in operations for the three months ended March 31, 2014 was \$10.2 million, due primarily to the net loss for the three months ended March 31, 2014 of \$15.9 million and the change in operating assets and liabilities of \$3.5 million. The change in operating assets and liabilities consisted of a decrease in receivables from GSK related to the collaboration agreement of \$0.7 million; a decrease of \$3.8 million in prepaid assets primarily related to Net Operating Loss (“NOL”) receivable; a decrease in accounts payable and accrued expenses of \$1.0 million related to program expenses.

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

Net cash provided by investing activities for the three months ended March 31, 2015 was \$21.7 million. Net cash provided by investing activities reflects \$40.7 million for the sale and redemption of marketable securities, partially offset by \$18.3 million for the purchase of marketable securities and \$0.8 million for the acquisition of property and equipment.

Net cash used in investing activities for the three months ended March 31, 2014 was \$5.8 million. Net cash used in investing activities reflects \$17.2 million for the purchase of marketable securities and \$40 thousand for the acquisition of property and equipment, partially offset by \$11.5 million from the sale and redemption of marketable securities.

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

Net cash provided by financing activities for the three months ended March 31, 2015 was \$4.1 million in proceeds from exercise of options.

Net cash used in financing activities for the three months ended March 31, 2014 was \$0.1 million for the payments of our secured loan agreement, partially offset by \$15 thousand in proceeds from exercise of options.

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

We do not anticipate that we will generate revenue from commercial sales until at least 2016, if at all. 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 2016.

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

#### *Milestone Payments / Royalties*

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, 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.

Under the Revised Agreement, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. for migalastat. 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 (“MSSM”) in addition to those owed to GSK.

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

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

Please refer to the section “*Recent Accounting Pronouncements*” under Footnote 2. Summary of Significant Accounting Policies, under 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 March 31, 2015, we held \$151.6 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 the fair value of our investments. 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., although we do conduct some clinical activities outside the U.S. While most expenses are paid in U.S. dollars, there are minimal payments made in local foreign currency. If exchange rates undergo a change of 10%, we do not believe that it would have a material impact on our results of operations or cash flows.

### **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

We are not a party to any material legal proceedings.

### ITEM 1A. RISK FACTORS

There have been no material changes with respect to the Risk Factors disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014.

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

#### Recent Sales of Unregistered Securities

None.

#### Issuer Purchases of Equity Securities

The Company did not purchase any shares of its common stock for the three months ended March 31, 2015.

### ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

### ITEM 4. MINE SAFETY DISCLOSURES

None.

### ITEM 5. OTHER INFORMATION

None.

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### ITEM 6. EXHIBITS

<u>Exhibit Number</u>	<u>Description</u>
3.1 (1)	Restated Certificate of Incorporation
3.2 (2)	Amended and Restated By-laws
10.1 (3)	First Amendment to Credit and Security Agreement, dated April , 2015 by and among Amicus Therapeutics, Inc. and the other entities shown as signatories thereto as a Borrower, the financial institutions or other entities from time to time parties as lenders, and Midcap Funding III Trust, as agent.
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 three months ended March 31, 2015, 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 Annual Report on Form 10K filed on February 28, 2012.

(2) Incorporated by reference to Exhibit 3.4 to our Registration Statement on Form S-1.

(3) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed April 27, 2015.

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**CERTIFICATIONS PURSUANT TO SECTION 302 OF  
THE SARBANES-OXLEY ACT OF 2002  
CERTIFICATION BY PRINCIPAL EXECUTIVE OFFICER**

I, John F. Crowley, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 5, 2015

/s/ John F. Crowley

John F. Crowley

**Chairman and Chief Executive Officer**

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**CERTIFICATIONS PURSUANT TO SECTION 302 OF  
THE SARBANES-OXLEY ACT OF 2002  
CERTIFICATION BY PRINCIPAL FINANCIAL OFFICER**

I, William D. Baird III, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 5, 2015

/s/ William D. Baird III

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William D. Baird III

**Chief Financial Officer**

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