

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended June 30, 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

Accelerated filer

Non-accelerated filer

Smaller Reporting Company

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of July 29, 2015 was 118,618,119 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), GALAFOLD™ 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 our pharmacological chaperone migalastat HCl (“Galafold™”);
- 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;
- our ability to obtain reimbursement for Galafold;
- our ability to commercialize Galafold in the European Union;
- 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.

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**PART I. FINANCIAL INFORMATION**

**Item 1. Financial Statements (unaudited)**

**Amicus Therapeutics, Inc.**  
**Consolidated Balance Sheets**  
*(Unaudited)*  
(in thousands, except share and per share amounts)

	June 30, 2015	December 31, 2014
<b>Assets:</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 249,023	\$ 24,074
Investments in marketable securities	112,396	127,601
Prepaid expenses and other current assets	3,578	2,902
<b>Total current assets</b>	<b>364,997</b>	<b>154,577</b>
Investments in marketable securities	—	17,464
Property and equipment, less accumulated depreciation of \$12,381 and \$11,520 at June 30, 2015 and December 31, 2014, respectively	3,379	2,811
In-process research & development	23,000	23,000
Goodwill	11,613	11,613
Other non-current assets	924	502
<b>Total Assets</b>	<b>\$ 403,913</b>	<b>\$ 209,967</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable and accrued expenses	\$ 16,901	\$ 16,345
Current portion of secured loan	—	3,840
<b>Total current liabilities</b>	<b>16,901</b>	<b>20,185</b>
Deferred reimbursements	36,620	36,620
Secured loan, less current portion	—	10,510
Contingent consideration payable	11,800	10,700
Deferred tax liability	9,186	9,186
Other non-current liability	504	588
<b>Commitments and contingencies</b>		
<b>Stockholders' equity:</b>		
Common stock, \$.01 par value, 250,000,000 shares authorized, 118,367,319 shares issued and outstanding at June 30, 2015, 125,000,000 shares authorized, 95,556,277 shares issued and outstanding at December 31, 2014	1,241	1,015
Additional paid-in capital	826,582	568,743
Accumulated other comprehensive income	(52)	(132)
Accumulated deficit	(498,869)	(447,448)
<b>Total stockholders' equity</b>	<b>328,902</b>	<b>122,178</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 403,913</b>	<b>\$ 209,967</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)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
<b>Revenue:</b>				
Research revenue	—	\$ 475	—	\$ 931
<b>Total revenue</b>	<b>—</b>	<b>475</b>	<b>—</b>	<b>931</b>

<b>Operating Expenses:</b>				
Research and development	\$ 17,234	\$ 9,978	\$ 33,347	\$ 19,970
General and administrative	8,278	4,753	14,705	9,929
Changes in fair value of contingent consideration payable	100	(305)	1,100	200
Restructuring charges	26	(81)	36	(89)
Loss on extinguishment of debt	952	—	952	—
Depreciation	353	396	861	808
Total operating expenses	26,943	14,741	51,001	30,818
Loss from operations	(26,943)	(14,266)	(51,001)	(29,887)
<b>Other income (expenses):</b>				
Interest income	158	36	329	78
Interest expense	(338)	(374)	(710)	(729)
Other expense	(10)	(10)	(39)	(19)
Net loss	\$ (27,133)	\$ (14,614)	\$ (51,421)	\$ (30,557)
Net loss per common shares — basic and diluted	\$ (0.27)	\$ (0.22)	\$ (0.53)	\$ (0.46)
Weighted-average common shares outstanding — basic and diluted	99,994,125	67,212,764	97,888,573	65,799,059

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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Net loss	\$ (27,133)	\$ (14,614)	\$ (51,421)	\$ (30,557)
<b>Other comprehensive income/(loss):</b>				
Unrealized (loss) gain on available-for-sale securities	(17)	(4)	80	(3)
Other comprehensive (loss) gain before income taxes	(17)	(4)	80	(3)
Provision for income taxes related to other (loss)/ comprehensive income items (a)	—	—	—	—
Other comprehensive (loss) income	\$ (17)	\$ (4)	\$ 80	\$ (3)
Comprehensive loss	\$ (27,150)	\$ (14,618)	\$ (51,341)	\$ (30,560)

(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)

	Six Months Ended June 30	
	2015	2014
<b>Operating activities</b>		
Net loss	\$ (51,421)	\$ (30,557)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	136	115
Depreciation	861	808
Stock-based compensation	4,191	2,748
Restructuring charges	36	(89)

Loss on extinguishment of debt	952	—
Non-cash changes in the fair value of contingent consideration payable	1,100	200
Changes in operating assets and liabilities:		
Receivable due from collaboration agreements	—	607
Prepaid expenses and other current assets	641	4,245
Other non-current assets	(482)	25
Accounts payable and accrued expenses	459	(155)
Non-current liabilities	(84)	14
Net cash used in operating activities	(43,611)	(22,039)
<b>Investing activities</b>		
Sale and redemption of marketable securities	63,163	31,114
Purchases of marketable securities	(30,414)	(28,849)
Purchases of property and equipment	(1,429)	(132)
Net cash provided by investing activities	31,320	2,133
<b>Financing activities</b>		
Proceeds from issuance of common stock, net of issuance costs	243,216	18,344
Payments of secured loan agreement	(15,291)	(199)
Proceeds from exercise of stock options	6,932	25
Purchase of vested restricted stock units	(1,617)	—
Proceeds from exercise of warrants	4,000	—
Net cash provided by financing activities	237,240	18,170
Net increase/(decrease) in cash and cash equivalents	224,949	(1,736)
Cash and cash equivalents at beginning of period	24,074	43,640
Cash and cash equivalents at end of period	\$ 249,023	\$ 41,904
<b>Supplemental disclosures of cash flow information</b>		
Cash paid during the period for interest	\$ 605	\$ 537

*See accompanying notes to consolidated financial statements*

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**Amicus Therapeutics, Inc.**

**Notes to Consolidated Financial Statements**

**Note 1. Description of Business**

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

Amicus Therapeutics, Inc. (the “Company,” “we,” “us,” or “our”) was incorporated on February 4, 2002 in Delaware and is a 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 the pharmacological chaperone migalastat HCl (“Galafold”), a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy (“ERT”) for Fabry disease. The Company’s development programs also include next-generation ERTs for LSDs, including Fabry disease, Pompe disease and Mucopolysaccharidosis Type I (“MPS I”). The Company’s activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development.

Our Fabry franchise strategy is to develop Galafold for all patients with Fabry disease - as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients.

During the first quarter of 2015, the Company met with regulatory authorities in Europe and the United States to discuss the approval pathways for migalastat as a monotherapy for Fabry patients who have amenable mutations. In June 2015, the European Medicines Agency (“EMA”) validated the Company’s Marketing Authorization Application (“MAA”) submission for Galafold and the Centralized Procedure has begun under Accelerated Assessment. The Committee for Medicinal Products for Human Use (“CHMP”) may shorten the MAA review period from 210 days, under standard review, to 150 days under Accelerated Assessment. The CHMP opinion is then reviewed by the European Commission, which generally issues a final decision on European Union (“EU”) approval within three months. The MAA submission will be reviewed in the Centralized Procedure, which if authorized, provides a marketing license valid in all 28 EU member states. Once authorized, the Company would then begin the country-by-country reimbursement approval process.

In the United States, the Company plans to conduct a pre-new drug application (“NDA”) meeting with the U.S. Food and Drug Administration (“FDA”) and to submit an NDA for Galafold under Subpart H (accelerated approval) in the second half of 2015 for accelerated approval. Following the MAA validation, the Company is also initiating the regulatory submission process in several additional geographies.

In June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share, with net proceeds of \$243.2 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

The Company had an accumulated deficit of approximately \$498.9 million at June 30, 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 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 2017.

## Note 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.

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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.

### *Significant Accounting Policies*

There have been no material changes to the Company’s significant accounting policies during the six months ended June 30, 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

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milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from the Company's activities to achieve the milestone, (ii) the milestone be related to past performance, and (iii) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved.

### ***Fair Value Measurements***

The Company records certain asset and liability balances under the fair value measurements as defined by the FASB guidance. Current FASB fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that market participants 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 ASU 2015-05, Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): *Customer's Accounting for Fees Paid in a Cloud Computing Arrangement*. The amendments in ASU 2015-05 provide guidance to customers about whether a cloud computing arrangement includes a software license. If a cloud computing arrangement includes a software license, then the customer should account for the software license element of the arrangement consistent with the acquisition of other software licenses. If a cloud computing arrangement does not include a software license, the customer should account for the arrangement as a service contract. The amendments do not change the accounting for a customer's accounting for service contracts. As a result of the amendments, all software licenses within the scope of Subtopic 350-40 will be accounted for consistent with other licenses of intangible assets. The ASU is effective for financial statements issued for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. Early adoption is permitted. We are currently assessing the impact that this standard will have on our consolidated financial statements.

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

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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.

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

As of June 30, 2015, the Company held \$249.0 million in cash and cash equivalents and \$112.4 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 are all classified as current unless indicated otherwise and consisted of the following as of June 30, 2015 and December 31, 2014 (in thousands):

	As of June 30, 2015			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	\$ 249,023	\$ —	\$ —	\$ 249,023
Corporate debt securities	103,101	2	(57)	103,046
Commercial paper	8,996	4	—	9,000
Certificate of deposit	350	—	—	350
	<u>\$ 361,470</u>	<u>\$ 6</u>	<u>\$ (57)</u>	<u>\$ 361,419</u>
Included in cash and cash equivalents	\$ 249,023	\$ —	\$ —	\$ 249,023
Included in marketable securities	112,447	6	(57)	112,396
Total cash and marketable securities	<u>\$ 361,470</u>	<u>\$ 6</u>	<u>\$ (57)</u>	<u>\$ 361,419</u>

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	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 six months ended June 30, 2015, unrealized holding gain of \$80 thousand and for the year ended December 31, 2014, unrealized holding loss of \$132 thousand, were included in the statement of comprehensive loss.

For the six months ended June 30, 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.

Unrealized loss positions in the available for sale securities as of June 30, 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 \$94.7 million and \$129.2 million as of June 30, 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 and six months, ended June 30, 2015 and 2014, were as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Balance, beginning	\$ (35)	\$ 2	\$ (132)	\$ 1
Current period changes in fair value, (a)	(17)	(4)	80	(3)
Reclassification of earnings, (a)	—	—	—	—
Balance, ending	<u>\$ (52)</u>	<u>\$ (2)</u>	<u>\$ (52)</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 with its lead ERT ATB200 for Pompe disease in late preclinical development. The acquisition of the Callidus assets and technology complements 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 June 30, 2015, approximately 25 thousand shares remain issuable to former Callidus stockholders. 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

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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 11.5% and various probability factors. As of June 30, 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 \$11.8 million at June 30, 2015, resulting in an increase in the contingent consideration payable and related expense of \$0.1 million and \$1.1 million for the three and six months ended June 30, 2015, respectively. 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.”

#### **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 six months ended June 30, 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 six months ended June 30, 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 June 30, 2015, the Company was authorized to issue 250 million shares of common stock. Dividends on common stock will be paid when, and if, declared by the board of directors. Each 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 June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share, with net proceeds of \$243.2 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

As of June 30, 2015, there was approximately \$1.4 million of receivables in other current assets on the balance sheet related to stock options exercises. The cash proceeds for these exercises was received in July 2015. The warrants issued in connection with the November 2013 securities and purchase agreement (“SPA”) were 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 were included in stockholder’s equity and were initially measured at fair value of \$1.0 million using the Black Scholes valuation model. The warrants were fully exercised in June 2015 resulting in cash proceeds to the Company of \$4.0 million.

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.

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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.

In November 2013, in connection with its acquisition of Callidus, 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 June 30, 2015, approximately 25 thousand shares remain issuable to former Callidus stockholders.

### 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 June 30, 2015 were approximately \$0.5 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 six months ended June 30, 2015. Deferral Plan assets as of June 30, 2015 were \$0.5 million and are classified as trading securities. The Deferred Plan assets are recorded at fair value with changes in the investments' fair value recognized in the period they occur. During the six months ended June 30, 2015, income from the investments was over \$6 thousand and unrealized loss was under \$10 thousand.

### 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 June 30,		Six Months ended June 30,	
	2015	2014	2015	2014
Expected stock price volatility	74.3%	81.3%	75.6%	81.4%
Risk free interest rate	1.7%	1.9%	1.7%	2.0%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

A summary of the Company's stock options for the six months ended June 30, 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	3,113.7	\$ 11.11		
Options exercised	(1,427.5)	\$ 5.83		
Options forfeited	(43.5)	\$ 5.10		
Balance at June 30, 2015	11,663.4	\$ 6.55	7.6 years	\$ 89.1
Vested and unvested expected to vest June 30, 2015	10,691.9	\$ 6.41	7.5 years	\$ 83.1
Exercisable at June 30, 2015	5,413.7	\$ 5.82	6.0 years	\$ 45.1

As of June 30, 2015, the total unrecognized compensation cost related to non-vested stock options granted was \$22.3 million and is expected to be recognized over a weighted average period of 3.2 years.

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#### Restricted Stock Units

A summary of non-vested Restricted Stock Units ("RSU") activity under the Plan for the six months ended June 30, 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	125	\$ 11.82		
Vested	(405)	\$ 10.80		
Forfeited	—	\$ —		
Non-vested units as of June 30, 2015	675	\$ 4.06	0.69	\$ 6.6
Non-vested units expected to vest at June 30, 2015	675	\$ 4.06	0.69	\$ 6.6

For the six months ended June 30, 2015, 0.4 million of the RSUs vested and all non-vested units are expected to vest over their normal term. The total fair value of restricted stock that vested and was released in the six months ended June 30, 2015 was \$4.4 million.

As of June 30, 2015, there was \$1.7 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 0.69 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Equity compensation expense recognized in:				
Research and development expense	\$ 1,044	\$ 702	\$ 1,991	\$ 1,252
General and administrative expense	1,187	788	2,200	1,496
Total equity compensation expense	<u>\$ 2,231</u>	<u>\$ 1,490</u>	<u>\$ 4,191</u>	<u>\$ 2,748</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 June 30, 2015. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended June 30, 2015.

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**Secured Debt**

As disclosed in “—Note 9. Short Term Borrowings and Long Term Debt”, the Company had a loan and security agreement with Midcap Financial, Oxford Finance and Silicon Valley Bank (“Term Loan”). The Company’s secured debt was classified as Level 2 and the fair value was 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 June 2015, the Company paid the outstanding balance on this loan in full.

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 \$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. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and hence classified as Level 3. In June 2015, EMA validated the submission of the Company’s MAA and the success fee became payable. The Company paid the success fee in connection with the re-payment of the debt in June 2015.

**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) the discount rate of 11.5% 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 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 June 30, 2015 were \$0.5 million, are classified as trading securities and recorded at fair value with changes in the investments’ fair value recognized in the period they occur. The assets investments consist of market exchanged mutual funds. During the six months ended June 30, 2015, the unrealized loss was under \$10 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.

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A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of June 30, 2015, are identified in the following table (in thousands):

	Level 1	Level 2	Total	
<b>Assets:</b>				
Cash/ money market funds	\$ 249,023	\$ —	\$ 249,023	
Corporate debt securities	—	103,046	103,046	
Commercial paper	—	9,000	9,000	
Certificate of deposit	—	350	350	
Deferred compensation plan assets	—	482	482	
	<u>\$ 249,023</u>	<u>\$ 112,878</u>	<u>\$ 361,901</u>	
<b>Liabilities:</b>				
Contingent consideration payable	—	—	11,800	11,800
Deferred compensation plan liability	—	490	—	490
	<u>\$ —</u>	<u>\$ 490</u>	<u>\$ 11,800</u>	<u>\$ 12,290</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	Level 3	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>

#### 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 bore interest at a rate per annum fixed at 8.5%. The Company made interest-only payments on the Term Loan from January 1, 2014. In June 2015, the Company paid off the outstanding balance of the term loan.

In connection with the repayment of the Term Loan, the Company paid a \$0.4 million exit fee. The Company also paid a \$0.4 million success fee due to the successful acceptance of the MAA in June 2015. The net loss on extinguishment of the debt was \$1.0 million and is included in the statement of operations for the six months ended June 30, 2015.

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#### 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 Galafold as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from Amicus to GSK. For Galafold 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 United States.

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

##### 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 disease. 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 six months ended June 30, 2015 and 2014, the Company recognized \$0 and \$0.9 million, respectively, 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.

#### 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 six months ended June 30, 2015 (in thousands):

	Balance as of December 31, 2014	Charges	Cash Payments	Adjustments	Balance as of June 30, 2015
Facilities consolidation	\$ 283	\$ —	\$ (130)	\$ 36	\$ 189

#### Note 12. Subsequent Events

The Company evaluated events that occurred subsequent to June 30, 2015 and there were no material recognized or non-recognized subsequent events during this period.

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#### 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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
<b>Historical</b>				
Numerator:				
Net loss attributable to common stockholders	\$ (27,133)	\$ (14,614)	\$ (51,421)	\$ (30,557)
Denominator:				
Weighted average common shares outstanding — basic and diluted	\$ 99,994,125	\$ 67,212,764	\$ 97,888,573	\$ 65,799,059

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

	As of June 30,	
	2015	2014
Options to purchase common stock	11,663	9,634
Outstanding warrants, convertible to common stock	—	1,600
Unvested restricted stock units	675	930
Total number of potentially issuable shares	12,338	12,164

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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 disease. Our development programs also include next-generation ERTs for LSDs, including Fabry disease, Pompe disease 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.

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 for all patients with Fabry disease - as a monotherapy for patients with amenable mutations (“Galafold™”) and in combination with ERT for all other patients.

#### *Galafold for Fabry Disease as a Monotherapy*

We have completed two Phase 3 global registration studies (Study 011 and Study 012) of Galafold 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 Galafold has resulted in reductions in disease substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations.

Study 011 was a 24-month study of Fabry disease patients naïve to or not receiving ERT, which investigated the safety and efficacy of oral Galafold. 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 Galafold 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 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 disease substrate (Globotriaosylceramide, or “GL-3”) in the interstitial capillaries of the kidney following treatment with oral Galafold (150 mg every other day). The study also measured clinical outcomes, including renal function, as secondary endpoints.

As previously reported, patients on Galafold 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 unbinding 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 Galafold 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 Galafold.

Results from this subgroup analysis further support use of the GLP HEK assay in predicting responsiveness to Galafold. Following a Type C Meeting with the U.S. Food and Drug Administration (“FDA”), we revised the Statistical Analysis Plan to pre-

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specify the primary analysis at month 12 as mean change in interstitial capillary 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 Galafold after month 6 demonstrated a statistically significant reduction in disease substrate, or kidney interstitial capillary GL-3, at month 12 ( $p=0.013$ ). Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.
- Six months migalastat treatment was associated with a significant reduction in plasma lyso-Gb3 versus placebo ( $p=.0033$ ). The reduction remained stable following 6 additional months migalastat. A significant reduction in plasma lyso-Gb3 was found in patients switching from placebo to migalastat between 6 and 12-months ( $p<.0001$ ).
- Kidney function, as measured by estimated glomerular filtration rate (“eGFR”) and iohexol measured GFR (“mGFR”), remained stable following 18-24 months of treatment with Galafold 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 Galafold 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 Galafold, 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.

Galafold 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 Galafold (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 Galafold extension phase. The study enrolled a total of 60 patients (males and females) with Fabry disease and genetic mutations identified as amenable to Galafold in the clinical trial assay. Subjects were randomized 1.5:1 to switch to Galafold 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 Galafold 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 Galafold 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:

- Galafold 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 disease, remained low and stable in patients with amenable mutations who switched from ERT to Galafold.
- There was a statistically significant decrease in LVMi from baseline to month 18 in patients who switched from ERT to Galafold.
- 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 Galafold.
- Galafold was generally safe and well-tolerated.

During the first quarter of 2015, we met with regulatory authorities in Europe and the United States to discuss the approval pathways for Galafold as a monotherapy for Fabry patients who have amenable mutations. In June 2015, the European Medicines

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Agency (“EMA”) validated our Marketing Authorization Application (“MAA”) submission for Galafold and the Centralized Procedure has begun under Accelerated Assessment. The Committee for Medicinal Products for Human Use (“CHMP”) may shorten the MAA review period from 210 days, under standard review, to 150 days under Accelerated Assessment. The CHMP opinion is then reviewed by the European Commission, which generally issues a final decision on EU approval within three months. The MAA submission will be reviewed in the Centralized Procedure, which if authorized, provides a marketing license valid in all 28 EU member states. Once authorized, Amicus would then begin the country-by-country reimbursement approval process.

In the United States, we plan to conduct a pre-NDA meeting with the FDA and to submit a New Drug Application (“NDA”) for Galafold under Subpart H (accelerated approval) in the second half of 2015. Following the MAA validation, the Company is also initiating the regulatory submission process in several additional geographies.

### *Migalastat Combination Programs for Fabry Disease*

In support of our Fabry Franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations, we plan to initiate 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 disease. We previously completed an open-label Phase 2 safety and pharmacokinetics study (“Study 013”) that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with agalsidase beta or agalsidase alfa in males with Fabry disease. 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.

### *Next-Generation ERT for Pompe Disease*

We are leveraging our biologics capabilities and CHART platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered 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.

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 preclinical studies, ATB200 demonstrated greater tissue enzyme levels and further substrate reduction compared to the currently approved ERT for Pompe disease (alglucosidase alfa), which were further improved with the addition of a chaperone. 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 disease. 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 increase GAA enzyme activity levels in plasma and muscle following co-administration 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 disease. 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 June 30, 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 June 30,		Six Months ended June 30,	
	2015	2014	2015	2014



### Third party direct project expenses

<b>Monotherapy Studies</b>								
Galafold™ (Fabry Disease — Phase 3)	\$	3,325	\$	2,906	\$	7,938	\$	5,820
<b>Combination (CHART) Studies</b>								
ATB200 + chaperone (Pompe Disease - Preclinical)		4,390		649		7,980		1,625
Migalastat + chaperone (Fabry Disease — Preclinical)		1,023		445		1,126		618
Neurodegenerative Diseases (Preclinical)		2		181		3		230
<b>Total third party direct project expenses</b>		<b>8,740</b>		<b>4,181</b>		<b>17,047</b>		<b>8,293</b>
<b>Other project costs (1)</b>								
Personnel costs		5,964		4,156		11,534		8,450
Other costs (2)		2,530		1,641		4,766		3,227
<b>Total other project costs</b>		<b>8,494</b>		<b>5,797</b>		<b>16,300</b>		<b>11,677</b>
<b>Total research and development costs</b>	<b>\$</b>	<b>17,234</b>	<b>\$</b>	<b>9,978</b>	<b>\$</b>	<b>33,347</b>	<b>\$</b>	<b>19,970</b>

- (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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Expected stock price volatility	74.3%	81.3%	75.6%	81.4%
Risk free interest rate	1.7%	1.9%	1.7%	2.0%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	\$ 0.00	\$ 0.00	\$ 0.00	\$ 0.00

#### *Restricted Stock Units*

In 2014 and 2015 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. In June 2015, 0.4 million RSU’s vested.

#### *Warrants*

The warrants issued in connection with our November 2013 securities and purchase agreement (“SPA”) were 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 were included in stockholder’s equity and were initially measured at fair value of \$1.0 million using the Black Scholes valuation model. These warrants were fully exercised in June 2015 resulting in net proceeds of \$4.0 million during the quarter.

#### *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

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

## Results of Operations

### *Three Months Ended June 30, 2015 Compared to Three Months Ended June 30, 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 June 30, 2015 and 2014, we recognized \$0 and \$0.5 million, respectively as Research Revenue for reimbursed research and development costs.

**Research and Development Expense.** Research and development expense was \$17.2 million during the three months ended June 30, 2015, representing an increase of \$7.2 million or 72.0% from \$10.0 million for the three months ended June 30, 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 \$1.8 million. Contract research increased by \$2.0 million and contract manufacturing by \$2.9 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the ATB200 + chaperone program, migalastat + chaperone program and the Galafold program.

**General and Administrative Expense.** General and administrative expense was \$8.3 million for the three months ended June 30, 2015, representing an increase of \$3.5 million or 72.9% from \$4.8 million for the three months ended June 30, 2014. The increase includes pre-commercial organization costs of \$2.5 million, and increases in personnel costs of \$1.3 million, recruitment of \$1.1 million and consulting fees of \$0.9 million. These increases were partially offset by decreases in legal expenses of \$0.1 million.

**Changes in Fair Value of Contingent Consideration Payable.** For three months ended June 30, 2015, we recorded expense of \$0.1 million representing an increase of \$0.4 million or 132.8% from (\$0.3) million for the three months ended June 30, 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.

**Loss from extinguishment of debt:** We recognized a loss of \$1.0 million in the second quarter of 2015 arising from the early extinguishment of the \$15 million secured loan. No such loss was recorded in the second quarter of 2014.

**Restructuring Charges.** Increase to the restructuring liability was \$26 thousand for three months ended June 30, 2015 as compared to a reduction of \$81 thousand for the three months ended June 30, 2014, and was due to the change in fair value of future minimum lease payments.

**Depreciation.** Depreciation expense was \$0.4 million for the three months ended June 30, 2015 and for the three months ended June 30, 2014.

**Interest Income.** Interest income was \$0.2 million for the three months ended June 30, 2015, representing an increase of \$0.1 million or 341.7% from \$36 thousand for the three months ended June 30, 2014. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

**Interest Expense.** Interest expense was approximately \$0.3 million for the three months ended June 30, 2015, as compared to \$0.4 million for the three months ended June 30, 2014. Interest expense was incurred on the \$15 million loan secured in December 2013. The interest expense was lower due to the payment of principal beginning in second quarter of 2015.

**Other Income/Expense.** Other income/expenses for the three months ended June 30, 2015 and June 30, 2014 was \$10 thousand and primarily included fair value changes of the success fee payable related to the \$15 million loan, and fair value changes to deferred compensation assets. The \$15 million term loan was paid in full during the second quarter of 2015.

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### *Six Months Ended June 30, 2015 Compared to Six Months Ended June 30, 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 six months ended June 30, 2015 and 2014, we recognized \$0 and \$0.9 million, respectively, as Research Revenue for reimbursed research and development costs.

**Research and Development Expense.** Research and development expense was \$33.3 million during the six months ended June 30, 2015, representing an increase of \$13.3 million or 66.5% from \$20.0 million for the six months ended June 30, 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 \$3.1 million. Contract research increased by \$4.3 million and contract manufacturing by \$4.9 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the ATB200 + chaperone program, migalastat + chaperone program and the Galafold program. The Galafold 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 months ended March 31, 2014.

*General and Administrative Expense.* General and administrative expense was \$14.7 million for the six months ended June 30, 2015, representing an increase of \$4.8 million or 48.5% from \$9.9 million for the six months ended June 30, 2014. The increase includes commercial organizational costs of \$3.1 million, and increases in consulting fees of \$1.7 million, personnel costs of \$1.6 million and recruitment of \$1.3 million.

*Changes in Fair Value of Contingent Consideration Payable.* For six months ended June 30, 2015, we recorded expense of \$1.1 million representing an increase of \$0.9 million or 4.5% from \$0.2 million for the six months ended June 30, 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.

*Loss from extinguishment of debt.* We recognized a loss of \$1.0 million in the six months ended June 30, 2015 arising from the early extinguishment of the \$15 million secured loan. No such loss was recorded in the six months ended June 30, 2014.

*Restructuring Charges.* Increase to the restructuring liability was \$36 thousand for six months ended June 30, 2015 as compared to a reduction in the liability of \$89 thousand for the six months ended June 30, 2014 and was due to the change in fair value of future minimum lease payments.

*Depreciation.* Depreciation expense was \$0.9 million for the six months ended June 30, 2015, representing an increase of \$0.1 million or 12.5% as compared to \$0.8 million for the six months ended June 30, 2014. The change was due to an increase in the amount of property, plant and equipment.

*Interest Income.* Interest income was \$0.3 million for the six months ended June 30, 2015, representing an increase of \$0.2 million or 200% from \$0.1 million for the six months ended June 30, 2014. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

*Interest Expense.* Interest expense was approximately \$0.7 million for the six months ended June 30, 2015 and June 30, 2014. Interest expense was incurred on the \$15 million loan secured in December 2013. The interest expense was lower due to the payment of principal beginning in second quarter of 2015.

*Other Expenses.* Other expenses for the six months ended June 30, 2015 included charges of \$39 thousand as compared to \$19 thousand for the six months ended June 30, 2014. The change was primarily from fair value changes of the success fee payable, related to the \$15 million loan that was fully paid off in June 2015 and from fair value changes to deferred compensation assets.

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## Liquidity and Capital Resources

### *Source of Liquidity*

In June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share. The offering generated gross proceeds of \$258.8 million. After deducting underwriting fees of \$15.5 million and other offering expenses of \$0.1 million, which included legal fees, the net proceeds of the offering were approximately \$243.2 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold (migalastat) for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

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 Galafold 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, \$576.3 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 paid the outstanding balance of the loan in the second quarter of 2015.

As of June 30, 2015, we had cash and cash equivalents and marketable securities of \$361.4 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances.

### *Net Cash Used in Operating Activities*

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

Net cash used in operations for the six months ended June 30, 2014 was \$22.0 million, due primarily to the net loss for the six months ended June 30, 2014 of \$30.6 million and the change in operating assets and liabilities of \$4.7 million. The change in operating assets and liabilities consisted of a decrease in receivables from collaboration agreements of \$0.6 million; a decrease of \$4.2 million in prepaid assets primarily related to Net Operating Loss (“NOL”) receivable; a decrease in accounts payable and accrued expenses of \$0.2 million related to program expenses.

### ***Net Cash Provided by Investing Activities***

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

Net cash provided by investing activities for the six months ended June 30, 2014 was \$2.1 million. Net cash provided by investing activities reflects \$31.1 million from the sale and redemption of marketable securities, partially offset by \$28.8 million for the purchase of marketable securities and \$0.1 million for the acquisition of property and equipment.

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

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

Net cash provided by financing activities for the six months ended June 30, 2014 was \$18.2 million. Net cash provided reflects \$18.3 million in net proceeds from sales of common stock under our ATM agreement with Cowen, partially offset by \$0.2 million for the payments of our secured loan agreement.

### ***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 Galafold;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development as well as the cost of 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;
- 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 2017.

### ***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 United States for Galafold. In addition, because we reacquired worldwide rights to Galafold, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement. We will owe royalties to Mt. Sinai School of Medicine in addition to those owed to GSK.

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

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

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

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At June 30, 2015, we held \$361.4 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on the fair value of our investments.

We have operated primarily in the United States, although we do conduct some clinical activities outside the United States. 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.

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## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

We are not a party to any material legal proceedings.

### ITEM 1A. RISK FACTORS

We have identified the following material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2014 (the “2014 Annual Report”). The risk factors listed below should be read in conjunction with the risk factors set forth in the 2014 Annual Report

*Even if we are able to commercialize Galafold or any other product candidate that we develop, the product may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.*

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

Our ability to commercialize Galafold or any other product candidate successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the EU and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for Galafold or any other product that we commercialize and, if coverage and reimbursement are available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for Galafold may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

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***If the FDA does not grant accelerated approval for Galafold, the timing and approval of the NDA will be significantly delayed.***

We plan to submit an NDA for accelerated approval (Subpart H) of Galafold with the FDA in the second half of 2015. Under the FDA's accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening disease or condition that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA has broad discretion over whether to grant approval based on a surrogate endpoint. Accordingly, even though we believe Galafold will meet the criteria for accelerated approval, the FDA may disagree and may determine not to grant such approval. If the FDA does not grant accelerated approval of Galafold, we will need to complete a Phase 3 clinical trial and will need to expend significantly more capital to obtain approval of Galafold.

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

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

If Galafold is approved for commercialization in Europe, we intend to market it in certain jurisdictions outside the United States. We expect that we will be subject to additional risks related to international operations or entering into international business relationships, including:

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

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

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

**Recent Sales of Unregistered Securities**

None.

## Issuer Purchases of Equity Securities

The following table sets forth purchases of our common stock for the three months ended June, 2015:

Period	(a) Total number of shares purchased	(b) Average Price Paid per Share	(c) Total number of shares purchased as part of publicly announced plans or programs	(d) Maximum number of shares that may yet be purchased under the plans or programs
April 1, 2015 – April 30, 2015	—	—	—	—
May 1, 2015 - May 31, 2015	149,776	\$ 10.80	—	255,224
June 1, 2015 – June 30, 2015	—	—	—	—
Total	<u>149,776</u>		<u>—</u>	

Pursuant to a restricted stock award dated April 10, 2014 between Amicus Therapeutics and certain employee recipients, certain employees were granted 405,000 restricted stock units, 50% of which vested on May 10, 2015. The remaining stock units vest on December 10, 2015, subject generally to the employee's continued employment with the Company. In order to comply with the minimum statutory federal tax withholding rate of 25%, 1.45% for Medicare plus 6.2% for Social Security where applicable, the employees surrendered to us a portion of their vested shares on the vesting date, representing between 26.45-32.65% of the total value of the shares then vested.

### 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

Exhibit Number	Description
1.1(1)	Underwriting Agreement dated June 11, 2015, by and amount Amicus Therapeutics, Inc., J.P. Morgan Securities LLC and Goldman, Sachs & Co., as representatives of the several underwriters set forth on Schedule I thereto.
3.1	Restated Certificate of Incorporation
3.2	Certificate of Amendment to the Company's Restated Certificate of Incorporation, as amended.
3.3 (2)	Amended and Restated By-laws
10.1 (3)	First Amendment to Credit and Security Agreement, dated April 27, 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 June 30, 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.

- (1) Incorporated by reference to Exhibit 1.1 to our Current Report on Form 8-K filed on June 12, 2015.
- (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 on April 28, 2015.

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# Delaware

PAGE 1

*The First State*

I, JEFFREY W. BULLOCK, SECRETARY OF STATE OF THE STATE OF DELAWARE, DO HEREBY CERTIFY THE ATTACHED ARE TRUE AND CORRECT COPIES OF ALL DOCUMENTS FILED FROM AND INCLUDING THE RESTATED CERTIFICATE OR A MERGER WITH A RESTATED CERTIFICATE ATTACHED OF "AMICUS THERAPEUTICS, INC." AS RECEIVED AND FILED IN THIS OFFICE.

THE FOLLOWING DOCUMENTS HAVE BEEN CERTIFIED:

RESTATED CERTIFICATE, FILED THE FIFTH DAY OF JUNE, A.D. 2007, AT 8:17 O'CLOCK A.M.


CERTIFICATE OF AMENDMENT, FILED THE SEVENTEENTH DAY OF JANUARY, A.D. 2012, AT 4:17 O'CLOCK P.M.



3457447 8100X

120209027

You may verify this certificate online  
at [corp.delaware.gov/authver.shtml](http://corp.delaware.gov/authver.shtml)

  
Jeffrey W. Bullock, Secretary of State  
AUTHENTICATION: 9383345

DATE: 02-22-12

RESTATED CERTIFICATE OF INCORPORATION  
OF  
AMICUS THERAPEUTICS, INC.

Incorporated pursuant to a Certificate of Incorporation initially filed  
with the Secretary of State of the State of Delaware on February 4, 2002  
Under the Name Amicus Therapeutics, Inc.

Amicus Therapeutics, Inc., a Delaware corporation (the "Corporation"), hereby certifies that this Restated Certificate of Incorporation has been duly adopted in accordance with the provisions of Sections 228, 242, and 245 of the General Corporation Law of the State of Delaware (the "DGCL"), and notice thereof has been given in accordance with the provisions of Section 228 of the DGCL:

**FIRST:** The name of the Corporation is Amicus Therapeutics, Inc.

**SECOND:** The address of the Corporation's registered office in the State of Delaware is c/o Corporation Trust Center, 1209 Orange Street, Wilmington, New Castle County, Delaware 19801. The registered agent in charge thereof is The Corporation Trust Company.

**THIRD:** The nature of the business and purposes to be conducted or promoted by the Corporation are as follows:

To engage in any lawful act or activity for which corporations may be organized under the General Corporation Law of the State of Delaware.

**FOURTH:** The total number of shares of all classes of capital stock which the Corporation shall have authority to issue is Sixty Million shares, consisting solely of:

Fifty Million (50,000,000) shares of common stock, par value \$.01 per share ("Common Stock");  
and

Ten Million (10,000,000) shares of preferred stock, par value \$.01 per share ("Preferred Stock").

The following is a statement of the powers, designations, preferences, privileges, and relative rights in respect of each class of capital stock of the Corporation.

A. COMMON STOCK.

1. General. The voting, dividend and liquidation rights of the holders of Common Stock are subject to and qualified by the rights of the holders of Preferred Stock.

2. Voting. The holders of Common Stock are entitled to one vote for each share held at all meetings of stockholders. There shall be no cumulative voting.

3. Dividends. Dividends may be declared and paid on the Common Stock from funds lawfully available therefor if, as and when determined by the board of directors of the Corporation (the "Board of Directors") and subject to any preferential dividend rights of any then outstanding shares of Preferred Stock.

4. Liquidation. Upon the dissolution or liquidation of the Corporation, whether voluntary or involuntary, holders of Common Stock will be entitled to receive all assets of the Corporation available for distribution to its stockholders, subject to any preferential rights of any then outstanding shares of Preferred Stock.

**B. PREFERRED STOCK.**

Shares of Preferred Stock may be issued from time to time in one or more series, each of such series to have such powers, designations, preferences, and relative, participating, optional, or other special rights, if any, and such qualifications and restrictions, if any, of such preferences and rights, as are stated or expressed in the resolution or resolutions of the Board of Directors providing for such series of Preferred Stock. Different series of Preferred Stock shall not be construed to constitute different classes of shares for the purposes of voting by classes unless expressly so provided in such resolution or resolutions.

Authority is hereby granted to the Board of Directors from time to time to issue the Preferred Stock in one or more series, and in connection with the creation of any such series, by resolution or resolutions to determine and fix the powers, designations, preferences, and relative, participating, optional, or other special rights, if any, and the qualifications and restrictions, if any, of such preferences and rights, including without limitation dividend rights, conversion rights, voting rights (if any), redemption privileges, and liquidation preferences, of such series of Preferred Stock (which need not be uniform among series), all to the fullest extent now or hereafter permitted by the DGCL. Without limiting the generality of the foregoing, the resolution or resolutions providing for the creation or issuance of any series of Preferred Stock may provide that such series shall be superior to, rank equally with, or be junior to the Preferred Stock of any other series, all to the fullest extent permitted by law. No resolution, vote, or consent of the holders of the capital stock of the Corporation shall be required in connection with the creation or issuance of any shares of any series of Preferred Stock authorized by and complying with the conditions of this Restated Certificate of Incorporation, the right to any such resolution, vote, or consent being expressly waived by all present and future holders of the capital stock of the Corporation.

Any resolution or resolutions adopted by the Board of Directors pursuant to the authority vested in them by this Article Fourth shall be set forth in a certificate of designation along with the number of shares of stock of such series as to which the resolution or resolutions shall apply and such certificate shall be executed, acknowledged, filed, recorded, and shall become effective, in accordance with §103 of the DGCL. Unless otherwise provided in any such resolution or resolutions, the number of shares of stock of any such series to which such resolution or resolutions apply may be increased (but not above the total number of authorized shares of the class) or decreased (but not below the number of shares thereof then outstanding) by a certificate likewise executed, acknowledged, filed and recorded, setting forth a statement that a specified increase or decrease therein has been authorized and directed by a resolution or resolutions likewise adopted by the Board of Directors. In case the number of such shares shall be decreased, the number of shares so specified in the certificate shall resume the status which they had prior to the adoption of the first resolution or resolutions. When no shares of any such class or series are outstanding, either because none were issued or because none remain outstanding, a certificate setting forth a

resolution or resolutions adopted by the Board of Directors that none of the authorized shares of such class or series are outstanding, and that none will be issued subject to the certificate of designations previously filed with respect to such class or series, may be executed, acknowledged, filed and recorded in the same manner as previously described and it shall have the effect of eliminating from the Restate Certificate of Incorporation all matters set forth in the certificate of designations with respect to such class or series of stock. If no shares of any such class or series established by a resolution or resolutions adopted by the Board of Directors have been issued, the voting powers, designations, preferences and relative, participating, optional or other rights, if any, with the qualifications, limitations or restrictions thereof, may be amended by a resolution or resolutions adopted by the Board of Directors. In the event of any such amendment, a certificate which (i) states that no shares of such class or series have been issued, (ii) sets forth the copy of the amending resolution or resolutions and (iii) if the designation of such class or series is being changed, indicates the original designation and the new designation, shall be executed, acknowledged, filed, recorded, and shall become effective, in accordance with §103 of the DGCL.

**FIFTH:** The following provisions are inserted for the management of the business and for the conduct of the affairs of the Corporation and for defining and regulating the powers of the Corporation and its directors and stockholders and are in furtherance and not in limitation of the powers conferred upon the Corporation by statute:

(a) The Board of Directors shall be divided into three classes of directors, as determined by the Board of Directors, such classes to be as nearly equal in number of directors as possible, having staggered three-year terms of office, the term of office of the directors of the first such class to expire as of the first annual meeting of the Corporation's stockholders following the closing of the Corporation's first public offering of shares of Common Stock registered pursuant to the Securities Act of 1933, as amended, those of the second class to expire as of the second annual meeting of the Corporation's stockholders following such closing, and those of the third class as of the third annual meeting of the Corporation's stockholders following such closing, such that at each annual meeting of stockholders after such closing, nominees will stand for election to succeed those directors whose terms are to expire as of such meeting. Any director serving as such pursuant to this paragraph (a) of Article FIFTH may be removed only for cause and only by the vote of the holders of a majority of the shares of the Corporation's stock entitled to vote for the election of directors.

(b) Except as the DGCL or the Corporation's by-laws may otherwise require, in the interim between annual meetings of stockholders or special meetings of stockholders called for the election of directors and/or for the removal of one or more directors and for the filling of any vacancy in that connection, any vacancies in the Board of Directors, including unfilled vacancies resulting from the removal of directors for cause, may be filled by the vote of a majority of the remaining directors then in office, although less than a quorum, or by the sole remaining director.

(c) If the office of any director becomes vacant by reason of death, resignation, disqualification, removal, failure to elect, or otherwise, the remaining directors, although more or less than a quorum, by a majority vote of such remaining directors may elect a successor or successors who shall hold office for the unexpired term.

(d) The Board of Directors shall have the power and authority: (i) to adopt, amend or repeal the Corporation's by-laws, subject only to such limitations, if any, as may be from time to time imposed by other provisions of this Restated Certificate of Incorporation, by law, or by the Corporation's by-laws; and (ii) to the full extent permitted or not prohibited by law, and without the consent of or other action by the stockholders, to authorize or create mortgages, pledges or other liens or encumbrances upon any or all of the assets, real, personal or mixed, and franchises of the Corporation, including after-acquired property, and to exercise all of the powers of the Corporation in connection therewith.

**SIXTH:** No director of the Corporation shall be personally liable to the Corporation or to any of its stockholders for monetary damages for breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability; provided, however, that to the extent required from time to time by applicable law, this Article Sixth shall not eliminate or limit the liability of a director, to the extent such liability is provided by applicable law, (i) for any breach of the director's duty of loyalty to the corporation or its stockholders, (ii) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (iii) under Section 174 of the DGCL, or (iv) for any transactions from which the director derived an improper personal benefit. No amendment to or repeal of this Article Sixth shall apply to or have any effect on the liability or alleged liability of any director for or with respect to any acts or omissions of such director occurring prior to the effective date of such amendment or repeal.

**SEVENTH:** The Corporation shall, to the fullest extent permitted by Section 145 of the DGCL and as further provided in its by-laws, each as amended from time to time, indemnify each person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative, by reason of the fact that he is or was, or has agreed to become, a director or officer of the Corporation, or is or was serving, or has agreed to serve, at the request of the Corporation, as a director, officer or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), judgements, fines and amounts paid in settlement actually and reasonably incurred by him or on his behalf in connection with such action, suit or proceeding and any appeal therefrom.

Indemnification may include payment by the Corporation of expenses in defending an action or proceeding in advance of the final disposition of such action or proceeding upon receipt of an undertaking by the person indemnified to repay such payment if it is ultimately determined that such person is not entitled to indemnification under this Article Seventh, which undertaking may be accepted without reference to the financial ability of such person to make such repayment.

The Corporation shall not indemnify any such person seeking indemnification in connection with a proceeding (or part thereof) initiated by such person unless the initiation thereof was approved by the Board of Directors.

The indemnification rights provided in this Article Seventh (i) shall not be deemed exclusive of any other rights to which those indemnified may be entitled under any law, agreement or vote of stockholders or disinterested directors or otherwise, and (ii) shall inure to the benefit of the heirs, executors and administrators of such persons. The Corporation may, to the extent authorized from time to time by its Board of Directors, grant indemnification rights to other

employees or agents of the Corporation or other persons serving the Corporation and such rights may be equivalent to, or greater or less than, those set forth in this Article Seventh.

**EIGHTH:** Whenever a compromise or arrangement is proposed between the Corporation and its creditors or any class of them and/or between the Corporation and its stockholders or any class of them, any court of equitable jurisdiction within the State of Delaware may, on the application in a summary way of the Corporation or of any creditor or stockholder thereof or on the application of any receiver or receivers appointed for the Corporation under the provisions of §291 of the DGCL; or on the application of trustees in dissolution or of any receiver or receivers appointed for the Corporation under the provisions of §279 of the DGCL, order a meeting of the creditors or class of creditors, and/or of the stockholders or class of stockholders of the Corporation, as the case may be, to be summoned in such a manner as the said court directs. If a majority of the number representing three-fourths (3/4ths) in value of the creditors or class of creditors, and/or of the stockholders or class of stockholders of the Corporation, as the case may be, agree to any compromise or arrangement and to any reorganization of the Corporation as a consequence of such compromise or arrangement, the compromise or arrangement and the said reorganization shall, if sanctioned by the court to which the said application has been made, be binding on all creditors or class of creditors, and/or stockholders or class of stockholders of the Corporation, as the case may be, and also on the Corporation.

**NINTH:** The Board of Directors, when considering a tender offer or merger or acquisition proposal, may take into account factors in addition to potential economic benefits to stockholders, including without limitation (i) comparison of the proposed consideration to be received by stockholders in relation to the then current market price of the Corporation's capital stock, the estimated current value of the Corporation in a freely negotiated transaction, and the estimated future value of the Corporation as an independent entity and (ii) the impact of such a transaction on the employees, suppliers, and customers of the Corporation and its effect on the communities in which the Corporation operates.

**TENTH:** Any action required or permitted to be taken by the stockholders of the Corporation may be taken only at a duly called annual or special meeting of the stockholders, and not by written consent in lieu of such a meeting, in which such action is properly brought before such meeting. Special meetings of stockholders may be called only by the Chairman of the Board of Directors, the President, or a majority of the Board of Directors.

**ELEVENTH:** The affirmative vote of the holders of at least sixty seven percent (67%) of the outstanding voting stock of the Corporation (in addition to any separate class vote that may in the future be required pursuant to the terms of any outstanding Preferred Stock) shall be required to amend or repeal the provisions of Articles Fourth (only to the extent it relates to the authority of the Board of Directors to issue shares of Preferred Stock in one or more series, the terms of which may be determined by the Board of Directors), Fifth, Seventh, Tenth, or Eleventh of this Restated Certificate of Incorporation or to reduce the numbers of authorized shares of Common Stock or Preferred Stock.

*[The remainder of this page is left intentionally blank.]*

Executed on June 5, 2007

AMICUS THERAPEUTICS, INC.

By: /s/ John F. Crowley  
Name: John F. Crowley  
Title: President

ACTIVE/1694892.2

STATE OF DELAWARE  
CERTIFICATE OF AMENDMENT  
OF CERTIFICATE OF INCORPORATION

The corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware does hereby certify:

**FIRST:** That at a meeting of the Board of Directors of  
Amicus Therapeutics, Inc.

resolutions were duly adopted setting forth a proposed amendment of the Certificate of Incorporation of said corporation, declaring said amendment to be advisable and calling a meeting of the stockholders of said corporation for consideration thereof. The resolution setting forth the proposed amendment is as follows:

**RESOLVED,** that the Certificate of Incorporation of this corporation be amended by changing the Article thereof numbered " FOURTH " so that, as amended, said Article shall be and read as follows:

FOURTH: The total number of shares of all classes of capital stock which the Corporation shall have authority to issue is One Hundred Thirty-Five Million (135,000,000) shares, consisting solely of: One Hundred Twenty-Five Million (125,000,000) shares of common stock, par value \$0.01 per share ("Common Stock"); and Ten Million (10,000,000) shares of preferred stock, par value \$0.01 per share ("Preferred Stock").

**SECOND:** That thereafter, pursuant to resolution of its Board of Directors, a special meeting of the stockholders of said corporation was duly called and held upon notice in accordance with Section 222 of the General Corporation Law of the State of Delaware at which meeting the necessary number of shares as required by statute were voted in favor of the amendment.

**THIRD:** That said amendment was duly adopted in accordance with the provisions of Section 242 of the General Corporation Law of the State of Delaware.

IN WITNESS WHEREOF, said corporation has caused this certificate to be signed this 17th day of January, 2012.

By:   
Authorized Officer

Title: Assistant Secretary

Name: Peter M. Macaluso  
Print or Type



**CERTIFICATE OF AMENDMENT  
TO THE  
RESTATED CERTIFICATE OF INCORPORATION  
OF  
AMICUS THERAPEUTICS, INC.**

AMICUS THERAPEUTICS, INC. (the "*Corporation*"), a corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware ("*DGCL*"), does hereby certify:

FIRST: That the Board of Directors of the Corporation (the "*Board*") duly adopted resolutions declaring advisable the amendment to the Restated Certificate of Incorporation of the Corporation (the "*Certificate*") and submitting the same to the stockholders of the Corporation for approval. The resolutions setting forth the proposed amendment are as follows:

**RESOLVED**, that the Certificate be amended by changing the Article thereof numbered "FOURTH" so that, as amended, said Article shall be and read as follows:

FOURTH: The total number of shares of all classes of capital stock which the Corporation shall have authority to issue is Two Hundred Sixty Million (260,000,000) shares, consisting solely of: Two Hundred Fifty Million (250,000,000) shares of common stock, par value \$.01 per share ("*Common Stock*") and Ten Million (10,000,000) shares of preferred stock, par value \$.01 per share ("*Preferred Stock*").

; and be it further

**RESOLVED**, that the Certificate of Amendment setting forth the foregoing resolutions be submitted to the stockholders of the Corporation for approval in accordance with Section 242 of the DGCL; and be it further

SECOND: That the stockholders of the Corporation approved the aforementioned amendment at a duly called meeting of stockholders held on June 10, 2015.

THIRD: That the foregoing amendment was duly adopted in accordance with the provisions of Section 242 of the DGCL.

*[Signature page follows]*

IN WITNESS WHEREOF, the undersigned, a duly appointed officer of Amicus Therapeutics, Inc., has executed this Certificate of Amendment on behalf of Amicus Therapeutics, Inc. this 10th day of June, 2015.

**AMICUS THERAPEUTICS, INC.**

By:  \_\_\_\_\_  
Name: William D. Baird, III  
Title: Chief Financial Officer

**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: August 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: August 5, 2015

/s/ William D. Baird III

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William D. Baird III

**Chief Financial Officer**

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