AMICUS THERAPEUTICS INC Form 10-Q May 03, 2016 Table of Contents

UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q



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

For the quarterly period ended March 31, 2016

OR

o 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 x No o

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 x No o

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 X Accelerated filer O

Non-accelerated filer O Smaller Reporting Company O

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

The number of shares outstanding of the registrant s common stock, \$.01 par value per share, as of April 26, 2016 was 127,049,901 shares.

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AMICUS THERAPEUTICS, INC.

Form 10-O for the Ouarterly Period Ended March 31, 2016

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We have filed applications to register certain trademarks in the U.S. and abroad, including Amicus Therapeutics@ and designs, At the forefront of therapies for rare and orphan diseases , Zorblisa , Galafold .

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This quarterly report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this quarterly report on Form 10-Q regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management are forward-looking statements. The words anticipate, believe, estimate, expect, potential, intend, may, plan, predict, project, will, should, would and similar expressions are if forward-looking statements, although not all forward-looking statements contain these identifying words.

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

- the progress and results of our clinical trials of our drug candidates;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new Fabry enzyme replacement therapy (ERT) cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage disorders (LSDs);
- the future results of on-going or later clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and obtain market acceptance of SD-101;
- the costs, timing and outcome of regulatory review of our product candidates including without limitation, the expected timing of the European Commission s final decision with respect to regulatory approval of migalastat in the European Union;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to obtain reimbursement for migalastat HCI;
- our ability to commercialize migalastat HClin 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;

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

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part I Item 1A Risk Factors of the Annual Report on Form 10-K for the year ended December 31, 2015, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

You should read this quarterly report on Form 10-Q in conjunction with the document that we reference herein. We do not assume any obligation to update any forward-looking statements.

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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)

| | 1 | March 31, 2016 | December 31, 2015 |
|---|----|-------------------|----------------------|
| Assets: | | | |
| Current assets: | | | |
| Cash and cash equivalents | \$ | 23,510 | \$ 69,485 |
| Investments in marketable securities | | 142,341 | 144,548 |
| Prepaid expenses and other current assets | | 2,662 | 2,568 |
| Total current assets | | 168,513 | 216,601 |
| Property and equipment, less accumulated depreciation of \$13,996 and \$13,353 at | | | |
| March 31, 2016 and December 31, 2015, respectively | | 8,413 | 6,178 |
| In-process research & development | | 486,700 | 486,700 |
| Goodwill | | 197,797 | 197,797 |
| Other non-current assets | | 1,484 | 1,108 |
| Total Assets | \$ | 862,907 | \$ 908,384 |
| Liabilities and Stockholders Equity | | | |
| Current liabilities: | | | |
| Accounts payable and accrued expenses | \$ | 22,501 | \$ 32,216 |
| Current portion of contingent consideration payable | | 41,926 | 41,400 |
| Total current liabilities | | 64,427 | 73,616 |
| Deferred reimbursements | | 35,756 | 35,756 |
| Due to related party | | 38,509 | 41,601 |
| Contingent consideration payable, less current portion | | 235,303 | 232,677 |
| Deferred tax liability | | 176,219 | 176,219 |
| Other non-current liabilities | | 1,061 | 681 |
| Commitments and contingencies | | | |
| Stockholders equity: | | | |
| Common stock, \$.01 par value, 250,000,000 shares authorized, 125,221,637 shares issued and outstanding at March 31, 2016, 250,000,000 shares authorized, 125,027,034 | | | |
| shares issued and outstanding at December 31, 2015 | | 1,308 | 1.306 |
| Additional paid-in capital | | 921,234 | 917,454 |
| Accumulated other comprehensive loss: | | 721,234 | 717,434 |
| Foreign currency translation adjustment | | (65) | |
| 2 oreign currency translation adjustment | | (03) | |

| Unrealized gain/ (loss) on available-for securities | 114 | (115) |
|---|------------|------------|
| Warrants | 12,298 | 8,755 |
| Accumulated deficit | (623,257) | (579,566) |
| Total stockholders equity | 311,632 | 347,834 |
| Total Liabilities and Stockholders Equity | \$ 862,907 | \$ 908,384 |

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Amicus Therapeutics, Inc.

Consolidated Statements of Operations

(Unaudited)

(in thousands, except share and per share amounts)

| | Three Months Ended March 31, | | | |
|--|---------------------------------|-------------|----|------------|
| | | 2016 | | 2015 |
| Operating Expenses: | | | | |
| Research and development | \$ | 23,425 | \$ | 16,113 |
| General and administrative | | 15,701 | | 6,427 |
| Changes in fair value of contingent consideration payable | | 3,152 | | 1,000 |
| Restructuring charges | | 50 | | 10 |
| Depreciation | | 673 | | 508 |
| Total operating expenses | | 43,001 | | 24,058 |
| Loss from operations | | (43,001) | | (24,058) |
| Other income (expenses): | | | | |
| Interest income | | 307 | | 171 |
| Interest expense | | (945) | | (372) |
| Other expense | | (52) | | (29) |
| Net loss | | (43,691) | | (24,288) |
| | | | | |
| Net loss attributable to common stockholders per common shares basic and diluted | \$ | (0.35) | \$ | (0.25) |
| Weighted-average common shares outstanding basic and diluted | | 125,178,517 | | 95,743,416 |

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Amicus Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss

(Unaudited)

(in thousands)

| Net loss attributable to common stockholders | \$ (43,691) | \$ (24,288) |
|---|----------------|----------------|
| Other comprehensive income/(loss): | | |
| Foreign currency translation adjustment | (65) | |
| Unrealized gain on available- for-sale securities | 229 | 97 |
| | | |
| Other comprehensive income | \$ 164 | \$ 97 |
| Comprehensive loss | \$ (43,527) | \$ (24,191) |

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Amicus Therapeutics, Inc.

Consolidated Statements of Cash Flows

(Unaudited)

(in thousands)

| | 2016 | Three Months Ended March 31, 2016 2015 | | |
|---|----------|--|----|----------|
| Operating activities | 2010 | | | 2010 |
| Net loss \$ | | (43,691) | \$ | (24,288) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | | | , , |
| Non-cash interest expense | | 451 | | 83 |
| Depreciation | | 673 | | 508 |
| Stock-based compensation | | 4,283 | | 1,960 |
| Restructuring charges | | 50 | | 10 |
| Non-cash changes in the fair value of contingent consideration payable | | 3,152 | | 1,000 |
| Changes in operating assets and liabilities: | | | | |
| Prepaid expenses and other current assets | | (94) | | 418 |
| Other non-current assets | | (377) | | (397) |
| Accounts payable and accrued expenses | | (9,763) | | (551) |
| Non-current liabilities | | 380 | | 282 |
| Net cash used in operating activities | (4 | 44,936) | | (20,975) |
| Investing activities | | | | |
| Sale and redemption of marketable securities | | 53,157 | | 40,739 |
| Purchases of marketable securities | (: | 50,721) | | (18,321) |
| Purchases of property and equipment | | (2,878) | | (753) |
| Net cash (used in)/ provided by investing activities | | (442) | | 21,665 |
| Financing activities | | | | |
| Purchase of vested restricted stock units | | (657) | | |
| Proceeds from exercise of stock options | | 155 | | 4,063 |
| Net cash (used in)/ provided by financing activities | | (502) | | 4,063 |
| Effect of exchange rate changes on cash and cash equivalents | | (95) | | |
| Net (decrease)/ increase in cash and cash equivalents | (4 | 45,975) | | 4,753 |
| Cash and cash equivalents at beginning of period | (| 69,485 | | 24,074 |
| Cash and cash equivalents at end of period \$ | <u>'</u> | 23,510 | \$ | 28,827 |
| Supplemental disclosures of cash flow information | | | | |
| Cash paid during the period for interest | | | \$ | 209 |

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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 and development- of advanced therapies to treat a range of devastating rare and orphan diseases. 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. SD-101, a product candidate in late-stage development, is a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa (EB). The Company is also leveraging its biologics and Chaperon-Advanced Replacement Therapy (CHART) platform technologies to develop next-generation ERT products for Fabry, Pompe, and other lysosomal storage disorders (LSDs). Our activities since inception have consisted principally of raising capital, establishing facilities, and performing research and development.

The Company s Fabry franchise strategy is to develop migalastat HCl (which the Company may refer to as migalastat) for all patients with Fabry disease - as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients. On April 1, 2016, the European Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion to approve the oral small molecule pharmacological chaperone migalastat as a first line therapy for Fabry disease in all patients who have an amenable genetic mutation. The label approved by the CHMP includes 269 Fabry-causing mutations which represent up to half of all patients with Fabry disease. A final decision from the European Commission is expected in the second quarter of 2016, after which the Company will begin the country-by-country reimbursement processes.

In February 2016, the Company entered into a sales agreement (the Sales Agreement) with Cowen and Company, LLC (Cowen), to create an at-the-market (ATM) equity program under which the Company from time to time may offer and sell shares of its common stock, par value \$0.01 per share, having an aggregate offering price of up to \$100,000,000 through Cowen as sales agent for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate of 3.0% of the gross proceeds per share sold through it as sales agent under the sales agreement. In April 2016, the Company sold 2.1 million shares of common stock under the ATM sales agreement resulting in net proceeds of \$16.2 million, which included Cowen s commission of \$0.5 million.

In October 2015, the Company entered into a Note and Warrant Purchase Agreement (the October 2015 Purchase Agreement) with Redmile Capital Fund, LP and certain of its affiliates (Redmile), whereby the Company sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its unsecured promissory notes (Notes) and (b) five-year warrants (Warrants) for 1.3 million shares of common stock. On February 19, 2016, the Company entered into a separate Note and Warrant Purchase Agreement (the February 2016 Purchase Agreement) with Redmile for \$50.0 million in unsecured promissory notes and five-year warrants for 1.9 million shares of common stock. The Company has agreed with Redmile to cancel the \$50 million note and warrants issued in October 2015 and pay only the accrued interest due. If certain clinical and regulatory milestones are met the Company may issue up to an additional \$25 million in unsecured promissory notes and a pro-rata amount of warrants. Based on the achievement of a positive CHMP opinion, \$10 million of the additional unsecured promissory notes are available which the Company plans to access in the second quarter of 2016. For additional information, see Note 9. Short-Term Borrowings and Long-Term Debt.

In September 2015, Amicus acquired Scioderm, Inc. (Scioderm), which strengthens the Company spipeline significantly with the addition of a novel, late-stage, proprietary topical cream and potential first-to-market therapy for EB. This investigational product was granted FDA breakthrough therapy designation in 2013 based on results from Phase 2 studies for the treatment of lesions in patients suffering with EB. SD-101 is currently being investigated in a Phase 3 study (SD-005) to support global regulatory submissions and was the first-ever treatment in EB clinical studies to show improvements in wound closure across all major EB subtypes.

For more details, refer to Note 4. Acquisitions.

In September 2015, a Pre-New Drug Application (NDA) meeting was held with the FDA to discuss the oral small molecule pharmacological chaperone migalastat HCl for the treatment of Fabry disease. Based on FDA feedback and subsequent follow-up interactions with the agency, the Company is further evaluating several U.S. pathways including a potential submission requesting Subpart H approval, or potentially generating additional data on migalastat HCl s effect on gastrointestinal symptoms in Fabry disease to support submission requesting full approval. Based on this updated guidance, the Company plans to meet with the FDA in mid-2016.

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The Company had an accumulated deficit of approximately \$623.3 million at March 31, 2016 and anticipates incurring losses through the fiscal year ending December 31, 2016 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from its initial public offering (IPO) and subsequent stock offerings, payments from partners during the terms of the collaboration agreements and other financing arrangements. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2017.

Note 2. Summary of Significant Accounting Policies

Principles of Consolidation

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

Basis of Presentation

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

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

Translation of Foreign Currencies

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

Use of Estimates

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

Concentration of Credit Risk

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

Significant Accounting Policies

There have been no material changes to the Company s significant accounting policies during the three months ended March 31, 2016, as compared to the significant accounting policies disclosed in Note 2 of the Consolidated Financial Statements in the Company s Annual Report on Form 10-K for the year ended December 31, 2015. However, the following accounting policies are the most critical in fully understanding and evaluating the Company s financial condition and results of operations.

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

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

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

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

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

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Fair Value Measurements

The Company records certain asset and liability balances under the fair value measurements as defined by the FASB guidance. Current FASB fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity s own assumptions that market participants assumptions would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

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

Contingent Liabilities

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

Recent Accounting Pronouncements

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

In March 2016, the FASB issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting.* The amendments are intended to improve the accounting for employee share-based payments and affect all organizations that issue share-based payment awards to their employees. Several aspects of the accounting for share-based payment award transactions are simplified, including: (a) income tax consequences; (b) classification of awards as either equity or liabilities; and (c) classification on the statement of cash flows. For public companies, the amendments are effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. For private companies, the amendments are effective for annual periods beginning after December 15, 2017, and interim periods within annual periods beginning after December 15, 2018. Early adoption is permitted for any organization in any interim or annual period. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In March 2016, the FASB issued ASU 2016-08, Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations (Reporting Revenue Gross versus Net). The amendments relate to when another party, along with the entity, is involved in providing a good or service to a customer. Topic 606 Revenue from Contracts with Customers requires an entity to determine whether the nature of its promise is to provide that good or service to the customer (i.e., the entity is a principal) or to arrange for the good or service to be provided to the customer by the other party (i.e., the entity is an agent). The amendments are

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intended to improve the operability and understandability of the implementation guidance on principal versus agent considerations. The effective date and transition of these amendments is the same as the effective date and transition of ASU 2014-09, *Revenue from Contracts with Customers* (Topic 606). Public entities should apply the amendments in ASU 2014-09 for annual reporting periods beginning after December 15, 2017, including interim reporting periods therein (i.e., January 1, 2018, for a calendar year entity). The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In February 2016, the FASB issued Accounting Standards Update (ASU) No. 2016-02, *Leases (Topic 842)*. Under the new guidance, lessees will be required recognize the following for all leases (with the exception of short-term leases) at the commencement date: (1) a lease liability, which is a lessee s obligation to make lease payments arising from a lease, measured on a discounted basis; and (2) a right-of-use asset, which is an asset that represents the lessee s right to use, or control the use of, a specified asset for the lease term. Under the new guidance, lessor accounting is largely unchanged. Certain targeted improvements were made to align, where necessary, lessor accounting with the lessee accounting model and Topic 606, *Revenue from Contracts with Customers*. The new lease guidance simplified the accounting for sale and leaseback transactions primarily because lessees must recognize lease assets and lease liabilities. Lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (i.e., January 1, 2019, for a calendar year entity). Early application is permitted for all public business entities and all nonpublic business entities upon issuance. Lessees (for capital and operating leases) and lessors (for sales-type, direct financing, and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition approach. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

Note 3. Cash, Money Market Funds and Marketable Securities

As of March 31, 2016, the Company held \$23.5 million in cash and cash equivalents and \$142.3 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.

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

| | As of March 31, 2016 | | | | | | | |
|---------------------------------------|----------------------|---------|----|------------|----|-----------|----|------------|
| | | | | Unrealized | Uı | nrealized | | |
| | | Cost | | Gain | | Loss | | Fair Value |
| Cash balances | \$ | 23,510 | \$ | | \$ | | \$ | 23,510 |
| Corporate debt securities | | 92,724 | | 29 | | (28) | | 92,725 |
| Commercial paper | | 49,081 | | 113 | | | | 49,194 |
| Certificate of deposit | | 422 | | | | | | 422 |
| | \$ | 165,737 | \$ | 142 | \$ | (28) | \$ | 165,851 |
| Included in cash and cash equivalents | \$ | 23,510 | \$ | | \$ | | \$ | 23,510 |
| Included in marketable securities | | 142,227 | | 142 | | (28) | | 142,341 |
| Total cash and marketable securities | \$ | 165,737 | \$ | 142 | \$ | (28) | \$ | 165,851 |

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

Unrealized gains and losses are reported as a component of other comprehensive income/ (loss) in the statements of comprehensive loss. For the three months ended March 31, 2016 and for the year ended December 31, 2015, unrealized holding gains of \$229 thousand and \$17 thousand, were included in the statements of comprehensive loss.

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

Unrealized loss positions in the available for sale securities as of March 31, 2016 and December 31, 2015 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months and as such are recognized in other comprehensive gain/ (loss). The fair value of these available for sale securities in unrealized loss positions was \$34.6 million and \$118.5 million as of March 31, 2016 and December 31, 2015, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company s balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income (AOCI) in the statements of comprehensive loss. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the three months, ended March 31, 2016 and 2015 were as follows (in thousands):

| | | Three Mor | led | |
|---|------|-----------|---------|-------|
| | 2016 | | 2015 | |
| Balance, beginning | \$ | (115) | \$ | (132) |
| Current period changes in fair value | | 229 | | 97 |
| Foreign currency translation adjustment | | (65) | | |
| Reclassification of earnings, | | | | |
| Balance, ending | \$ | 49 | \$ | (35) |

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|--------------|----|----------|
| | | |

Note 4. Acquisitions

Acquisition of Scioderm, Inc.

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

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

The fair value of the contingent consideration payments on the acquisition date was \$259.0 million. This was an estimate based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a range of discount rates between 0.4% and 1.1% as interpolated from the U.S. Treasury constant maturity yield curve over the time frame for clinical and regulatory milestones and a range of discount rates between 1.0% and 2.2% for revenue-based milestones. The range of outcomes and assumptions used to develop these estimates have been updated to better reflect the probability of certain milestone outcomes as of March 31, 2016 (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 \$259.9 million at March 31, 2016, of which \$35.9 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of \$2.2 million for the three months ended March 31, 2016. The expense is recorded with the change in fair value of contingent consideration payable as part of the operating expense line item in the Consolidated Statement of Operations. See Note 8. Assets and Liabilities Measured at Fair Value , for additional discussion regarding fair value measurements of the contingent acquisition consideration payable.

For additional information, see Note 5. Goodwill and Intangible Assets.

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

| There were | e no acqui | isition-related | transaction coa | sts recognized | in 2016. |
|------------|------------|-----------------|-----------------|----------------|----------|
| | | | | | |

Acquisition of Callidus Biopharma, Inc.

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

The fair value of the contingent acquisition consideration payments on the acquisition date was \$10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. As of March 31, 2016, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes; see

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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 \$17.3 million at March 31, 2016, of which \$6.0 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of \$1.0 million for the three months ended March 31, 2016. The expense is recorded as part of operating expense in the Consolidated Statement of Operations

For further information, see Note 5. Goodwill and Intangible Assets.

Note 5. Goodwill and Intangible Assets

In connection with the acquisitions discussed in Note 4. Acquisitions , the Company has recognized goodwill of \$197.8 million. The following table represents the changes in goodwill for the three months ended March 31, 2016:

| | (in n | nillions) |
|------------------------------|--------|-----------|
| Balance at December 31, 2015 | \$ | 197.8 |
| Change in goodwill | | |
| Balance at March 31, 2016 | \$ | 197.8 |

In connection with the acquisitions discussed in Note 4. Acquisitions, the Company recognized IPR&D of \$486.7 million. Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. The following table represents the changes in IPR&D for the three months ended March 31, 2016:

| | (in m | illions) |
|------------------------------|--------|----------|
| Balance at December 31, 2015 | \$ | 486.7 |
| Change in IPR&D | | |
| Balance at March 31, 2016 | \$ | 486.7 |

Goodwill and intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. For the three months ended March 31, 2016, there were no indicators of impairment.

Note 6. Debt Instruments and Related Party Transactions

In October 2015, the Company entered into a Note and Warrant Purchase Agreement (the October 2015 Purchase Agreement) with Redmile Capital Fund, LP and certain of its affiliates, whereby it sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its unsecured promissory notes (Notes) and (b) five-year warrants (Warrants) for 1.3 million shares of Common Stock. The payment terms under the purchase agreement contains two installments, the first \$15.0 million in October 2017 and the balance \$35.0 million in October 2020. Interest was payable at 4.1% on a monthly basis over the term of the loan. The promissory notes are recorded as due to related party on the consolidated balance sheets. Due to the embedded redemption (put and/or call) features in the note agreement, it was determined that the fair value of the warrants should be bifurcated from the value of the notes payable and recorded as a debt discount. The relative fair value of the warrants and the debt discount as related to the October 2015 purchase agreement was determined to be \$8.8 million.

On February 19, 2016, the Company entered into a Note and Warrant Purchase Agreement (the February 2016 Purchase Agreement) with Redmile for an aggregate amount of up to \$75.0 million. The Company has agreed with Redmile that in full consideration of the purchase price for the notes issued under the February 2016 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purchase Agreement and the Company paid Redmile the interest accrued thereunder. As of March 31, 2016, Redmile beneficially owned approximately 10.6% of the Company s outstanding shares of common stock. As such the promissory notes are presented as due to related party on the consolidated balance sheets.

Pursuant to the February 2016 agreement, at closing, it sold, on a private placement basis (a) \$50.0 million aggregate principal amount of unsecured promissory notes (Initial Notes) and (b) five year warrants to purchase up to 37 shares of Amicus common stock, par value \$0.01 per share (Common Stock) for every \$1,000 of the principal amount of Initial Notes purchased (Initial Warrants), for an aggregate of up to 1,850,000 shares of common stock issuable under the Initial Warrants. The payment terms contain two installments, the first \$15.0 million in October 2017 and the balance \$35.0 million in October 2021. The interest rate was is 3.875% and payable upon of maturity.

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This transaction was accounted for as a debt modification in accordance with ASC 470-50. The fees paid to third parties in conjunction with this transaction were de minimus. The incremental fair value between the warrants that were cancelled and the February issued warrants of \$3.5 million were recorded as additional unamortized debt discount on the balance sheet and added to the prior warrant balance within equity. The debt discount will be amortized over the life of the Initial Notes using the effective interest rate method. In order to arrive at the incremental fair value of the warrants issued in February, we utilized the Black-Scholes valuation model using the following six inputs: (1) the closing price of Amicus stock on the day of evaluation of \$6.77; (2) the exercise price of the warrants of \$7.95; (3) the remaining term of the warrants of 5 years; (4) the volatility of Amicus stock for the five year term of 84.94%; (5) the annual rate of dividends of 0%; and (6) the risk-free rate of return of 1.24%. The fair value of the warrants was \$8.0 million.

As of March 31, 2016, the warrants were recorded at \$12.3 million and the notes due to related party are recorded at \$38.5, net of discount of \$11.5 million. The debt discount amortization for the three months ended March 31, 2016 was \$0.5 million.

Note 7. Stockholders Equity

Common Stock and Warrants

As of March 31, 2016, the Company was authorized to issue 250 million shares of common stock. Dividends on common stock will be paid when, and if, declared by the board of directors. Each 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.

On February 26, 2016, the Company entered into the Sales Agreement with Cowen to create an at-the-market equity program under which the Company from time to time may offer and sell shares of its common stock, par value \$0.01 per share, having an aggregate offering price of up to \$100 million through Cowen (the ATM Facility). Sales of the shares under the Sales Agreement were to be made in transactions that were deemed to be at the market offerings as defined in Rule 415 promulgated under the Securities Act of 1933, as amended, including sales made directly on The NASDAQ Global Market, on any other existing trading market for the common stock or to or through a market maker. In addition, with the Company's prior written approval, Cowen may also sell shares of common stock by any other method permitted by law, including in negotiated transactions. Cowen will act as sales agent using its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of The NASDAQ Stock Market LLC. There is no arrangement for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate up to 3.0% of the gross proceeds per share sold through it as sales agent under the sales agreement. In April 2016, the Company sold 2.1 million shares of common stock under the ATM sales agreement resulting in net proceeds of \$16.2 million, which included Cowen's commission of \$0.5 million.

In October 2015, the Company entered into the October 2015 Purchase Agreement with Redmile, whereby the Company sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its Notes and (b) Warrants for 1.3 million shares of common stock. On February 19, 2016, the Company entered into the February 2016 Purchase Agreement with Redmile for \$50.0 million in unsecured promissory notes and five-year warrants for 1.9 million shares of common stock. The Company has agreed with Redmile to cancel the \$50 million note and warrants issued in October 2015 and pay only the accrued interest due of \$0.8 million. If certain clinical and regulatory milestones are met the Company may issue up to an additional \$25 million in unsecured promissory notes and a pro-rata amount of warrants. In accordance with ASC 470, the transactions qualifies as a modification of debt.

The warrants are valued at issuance date using the Black-Scholes valuation model using the following six inputs: (1) the closing price of the Company s stock on the day of evaluation of \$6.77; (2) the exercise price of the warrants of \$7.95; (3) the remaining term of the warrants of 5 years; (4) the volatility of the Company s stock for the five year term of 84.94%; (5) the annual rate of dividends of 0%; and (6) the risk-free rate of return of 1.24%. The Black Scholes value of the warrants was \$8.0 million with an incremental fair value of \$3.5 million, resulting in a closing balance of the warrants of \$12.3 million as of March 31, 2016.

Nonqualified Cash Plan

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

Deferred compensation amounts under the Deferral Plan as of March 31, 2016 were approximately \$1.0 million, as compared to \$0.7 million on December 31, 2015 and are included in other long-term liabilities. Deferral Plan assets as of March 31, 2016 were \$1.0 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 three months ended March 31, 2016, income from the investments was \$2 thousand and unrealized loss was \$8 thousand.

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Equity Incentive Plan

Stock Option Grants

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

| | | Three Months ended March 31, | | | | | |
|------------------------------------|------|---------------------------------|----|------|-------|--|--|
| | 2016 | í | | 2015 | | | |
| Expected stock price volatility | | 81.2% | | | 77.1% | | |
| Risk free interest rate | | 1.8% | | | 1.7% | | |
| Expected life of options (years) | | 6.25 | | | 6.25 | | |
| Expected annual dividend per share | \$ | 0.00 | \$ | | 0.00 | | |

A summary of the Company s stock options for the three months ended March 31, 2016 is as follows:

| | Number of Shares (in thousands) | Weighted Average Exercise Price | Weighted Average Remaining Contractual Life | A | ggregate Intrinsic Value (in millions) |
|---|--|---------------------------------------|--|----|---|
| Balance at December 31, 2015 | 11,729.2 | \$ 7.11 | | | |
| Options granted | 2,570.5 | \$ 8.83 | | | |
| Options exercised | (47.0) | \$ 3.92 | | | |
| Options forfeited | (150.4) | \$ 8.65 | | | |
| Balance at March 31, 2016 | 14,102.3 | \$ 7.42 | 7.6 years | \$ | 31.3 |
| Vested and unvested expected to vest March 31, 2016 | 13,074.9 | \$ 7.29 | 7.5 years | \$ | 30.3 |
| Exercisable at March 31, 2016 | 6,273.6 | \$ 5.77 | 5.8 years | \$ | 19.9 |

As of March 31, 2016, the total unrecognized compensation cost related to non-vested stock options granted was \$33.7 million and is expected to be recognized over a weighted average period of 3.2 years.

Restricted Stock Units

A summary of non-vested Restricted Stock Units (RSU) activity under the Plan for the three months ended March 31, 2016 is as follows:

| | Number of Shares (in thousands) | Weighted Average Grant Date Fair Value | Weighted Average Remaining Years | Aggregate Intrinsic Value (in millions) | |
|--|-------------------------------------|---|--|--|---|
| Non-vested units as of December 31, 2015 | 478.5 | \$ 10.38 | | | |
| Granted | | \$ | | | |
| Vested | | \$ | | | |
| Forfeited | | \$ | | | |
| | | | | | |
| Non-vested units as of March 31, 2016 | 478.5 | \$ 10.38 | 1.42 | \$ 0.7 | 1 |
| Non-vested units expected to vest at March 31, | | | | | |
| 2016 | 478.5 | \$ 10.38 | 1.42 | \$ 0.7 | , |

For the three months ended March 31, 2016, none of the RSUs vested and all non-vested units are expected to vest over their normal term.

As of March 31, 2016, there was \$3.5 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 1.42 year.

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Compensation Expense Related to Equity Awards

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

| | | Three Months Ended March 31, | | | | |
|--|------|------------------------------|----|-------|--|--|
| | 2016 | | | 2015 | | |
| Equity compensation expense recognized in: | | | | | | |
| Research and development expense | \$ | 1,936 | \$ | 947 | | |
| General and administrative expense | | 2,347 | | 1,013 | | |
| Total equity compensation expense | \$ | 4,283 | \$ | 1,960 | | |
| | | | | | | |
| Tomoquity componential expense | Ψ | .,_00 | Ψ | 1,200 | | |

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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 months ended March 31, 2016. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 2016.

Note Payable to Related Party

In connection with the notes payable to Redmile, as disclosed in Note 6. Debt Instruments and Related Party Transactions , and Warrants as disclosed in Note 7. Stockholders Equity, the Company recorded the notes as a liability of \$38.5 million on an amortized cost basis.

The Company evaluated the warrants issued in relation to the February 2016 Purchase Agreement against current accounting guidance and determined that the related warrants should be accounted as a component of equity. As such, these warrants which are considered Level 3 instruments, were valued at the issuance date using the Black-Scholes valuation model using the following six inputs: (1) the closing price of the Company s stock on the day of evaluation at \$6.77; (2) the exercise price of the warrants at \$7.95; (3) the remaining term of the warrants at 5 years; (4) the volatility of the Company s stock for the five year term at 84.94%; (5) the annual rate of dividends at 0%; and (6) the risk-free rate of return at 1.24%. The Black-Scholes value of the warrants was \$12.3 million.

As of March 31, 2016, the warrants are recorded at \$12.3 million and the notes due to related party are recorded at \$38.5 million, net of discount of \$11.5 million.

Contingent Consideration Payable

The contingent consideration payable resulted from the acquisitions of Scioderm and Callidus, as discussed in Note 4. Acquisitions. 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. The valuation is performed quarterly. Gains and losses are included in the statement of operations.

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

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

| Contingent Consideration Liability | Fair value as of March 31, 2016 | Valuation Technique | Unobservable Input | Range |
|---------------------------------------|------------------------------------|---|--|------------|
| | | | Discount rate | 0.5%-1.0% |
| Clinical and regulatory milestones | \$ 238.1 million | Probability weighted discounted cash flow | Probability of achievement of milestones | 66.5% -70% |
| | | | Projected year of payments | 2016-2019 |
| | | | | |
| | | | Revenue volatility | 58% |
| Revenue-based milestones | \$ 21.8 million | Monte Carlo | Discount rate | 0.8%-1.9% |
| | | | Projected year of payments | 2018-2028 |

The following significant unobservable inputs were used in the valuation of the contingent consideration payable of Callidus:

| Contingent Consideration Liability | Fair value as of March 31, 2016 | Valuation Technique | Unobservable Input | Range |
|---------------------------------------|---------------------------------|---|--|-----------|
| | | | Discount rate | 10.5% |
| Clinical and regulatory milestones | \$ 17.3 million | Probability weighted discounted cash flow | Probability of achievement of milestones | 2.5%-100% |
| | | | Projected year of payments | 2016-2026 |

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

The following table shows the change in the balance of contingent consideration payable for the three months ended March 31, 2016 and 2015, respectively:

| 016 | | 2015 |
|---------|---------|--------|
| | 2015 | |
| 274,077 | \$ | 10,700 |
| 3,152 | | 1,000 |
| | ,,,,,,, | , |

Balance, end of the period \$ 277,229 \$ 11,700

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Deferred Compensation Plan- Investment and Liability

As disclosed in Note 7. Stockholders Equity , the Deferral Plan provides certain key employees and members of the Board of Directors with an opportunity to defer the receipt of such participant s base salary, bonus and director s fees, as applicable. Deferral Plan assets as of March 31, 2016 were \$1.0 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 asset investments consist of market exchanged mutual funds. During the three months ended March 31, 2016, the unrealized loss was \$8 thousand. The Company considers its investments in marketable securities, as available-for-sale and classifies these assets and related liability within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities.

A summary of the fair value of the Company s assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2016, are identified in the following table (in thousands):

| | Level 1 | Level 2 | Total |
|-------------------------------|--------------|---------------|---------------|
| Assets: | | | |
| Cash/ money market funds | \$ 23,510 | \$ | \$ 23,510 |
| Corporate debt securities | | 92,725 | 92,725 |
| Commercial paper | | 49,194 | 49,194 |
| Certificate of deposit | | 422 | 422 |
| Market exchanged mutual funds | | 1,035 | 1,035 |
| | \$ 23,510 | \$ 143,376 | \$ 166,886 |

| | Level 2 | | Level 3 | Total |
|--------------------------------------|-------------|-------|---------------|---------------|
| Liabilities: | | | | |
| Contingent consideration payable | | | 277,229 | 277,229 |
| Deferred compensation plan liability |] | 1,043 | | 1,043 |
| | \$ 1 | 1,043 | \$ 277,229 | \$ 278,272 |

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

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

| | Level 2 | Level 3 | Total |
|----------------------------------|---------|---------|---------|
| Liabilities: | | | |
| Contingent consideration payable | | 274,077 | 274,077 |

| Deferred compensation plan liability | 667 | | 667 |
|--------------------------------------|-----------|---------------|---------------|
| | \$ 667 | \$ 274,077 | \$ 274,744 |
| | | | |
| | 21 | | |
| | 21 | | |

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Note 9. Restructuring Charges

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

The following table summarizes the restructuring charges and utilization for the three months ended March 31, 2016 (in thousands):

| | Bala | ance as of | | | | | | Balance as of | |
|--------------------------|-------|--------------|---------|-----|------------|--------------|------------|----------------|---|
| | Decem | ber 31, 2015 | Charges | Cas | h Payments | Fair Value A | djustments | March 31, 2016 | |
| Facilities consolidation | \$ | 118 | \$ | \$ | (88) | \$ | 50 | \$ 80 |) |

Note 10. 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:

| | | Three Mo Mar | nths End ch 31, | ed |
|--|-------------------|-------------------|--------------------|------------|
| (In thousands, except per share amounts) | | 2016 | | 2015 |
| Historical | | | | |
| Numerator: | | | | |
| Net loss attributable to common stockholders | | \$ (43,691) | \$ | (24,288) |
| Denominator: | | | | |
| Weighted average common shares outstanding | basic and diluted | \$ 125,178,517 | \$ | 95,743,416 |

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

As of March 31,

| | 2016 | 2015 |
|---|--------|--------|
| Options to purchase common stock | 14,102 | 10,603 |
| Outstanding warrants, convertible to common stock | 1,850 | 1,600 |
| Unvested restricted stock units | 479 | 955 |
| Total number of potentially issuable shares | 16,431 | 13,158 |

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Note 11. Commitments and Contingencies

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company s common stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. The Company anticipates that these lawsuits will be consolidated into a consolidated action.

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

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

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

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

Note 12. Subsequent Events

As previously reported, on August 30, 2015, we entered into an Agreement and Plan of Merger with Scioderm, which was subsequently amended and modified (the Merger Agreement). A payment in the amount of \$5 million was made because the first milestone under the Merger

Agreement was achieved. The company will evaluate the accounting impact, if any, on the contingent consideration payable in the second quarter of 2016. See Note 4. Acquisitions for further information regarding any potential future payments related to the Scioderm merger.

In April 2016, the Company sold 2.1 million shares of common stock under the ATM sales agreement resulting in net proceeds of \$16.2 million, which included Cowen s commission of \$0.5 million.

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

Overview

We are a global, late-stage, patient-focused biotechnology company engaged in the discovery and development of a diverse set of novel treatments for patients living with devastating rare and orphan diseases. Our lead product candidate, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement of therapy (ERT) for Fabry disease. SD-101, a product candidate in late-stage development, is a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis (EB). We are also leveraging our Chaperon-Advanced Replacement Therapy (CHART) platform technologies to develop next-generation ERT products for Fabry disease, Pompe disease and other lysosomal storage disorders (LSDs). We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

Program Status

We have completed two global Phase 3 registration studies of our lead product candidate, migalastat HCl, an orally administered small molecule pharmacological chaperone for the treatment of Fabry disease, an LSD. On April 1, 2016, the European Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion to approve the oral small molecule pharmacological chaperone migalastat as a first line therapy for Fabry disease in all patients who have an amenable genetic mutation. The label approved by the CHMP includes 269 Fabry-causing mutations which represent up to half of all patients with Fabry disease. A final decision from the European Commission (EC) is expected in the second quarter of 2016, after which we will begin the country-by-country reimbursement processes. In the U.S., we plan to meet with the U.S. Food and Drug Administration (FDA) in mid-2016 to discuss certain additional data regarding migalastat HCl and a potential pathway to submit a New Drug Application (NDA) for migalastat HCl in the U.S.

We are also in Phase 3 clinical development of a novel topical cream, SD-101, for the treatment of the genetic connective tissue disorder EB, for which no other pharmacological therapies are currently approved. We have also initiated a clinical study in patients with Pompe disease, another LSD to investigate our novel treatment paradigm that consists of ATB200, a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure to enhance uptake, co-administered with a pharmacological chaperone, AT2221, to improve activity and stability. Leveraging our biologics capabilities and platform technologies, we have the potential to develop additional novel ERTs for Fabry disease and other LSDs. We believe that our platform technologies and our advanced product pipeline uniquely position us at the forefront of developing therapies to potentially address significant unmet needs for devastating rare and orphan diseases.

| Migalastat | for | Fabry | Disease |
|------------|-----|-------|---------|
|------------|-----|-------|---------|

Overview

Our most advanced product candidate, migalastat, is an investigational, small molecule pharmacological chaperone for the treatment of Fabry disease. As an orally administered monotherapy, migalastat is designed to bind to and stabilize an endogenous alpha-galactosidase A (alpha-Gal A) enzyme in those patients with genetic mutations identified as amenable in a GLP cell-based amenability assay. In preclinical and clinical studies, we are also evaluating the use of migalastat in combination with a novel Fabry ERT for patients who have non-amenable genetic mutations

Migalastat for Fabry Disease as a Monotherapy: Program Overview and Regulatory Status

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

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

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On April 1, 2016, the CHMP adopted a positive opinion to approve the oral small molecule pharmacological chaperone migalastat as a first line therapy for Fabry disease in all patients who have an amenable genetic mutation. The label approved by the CHMP includes 269 Fabry-causing mutations which represent up to half of all patients with Fabry disease. A final decision from the EC is expected in the second quarter of 2016, after which we will begin the country-by-country reimbursement processes.

Migalastat in Combination with ERT for Fabry Disease

We are internally developing our own Fabry cell line for co-formulation with migalastat as a novel treatment paradigm 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 enzymes in the circulation in any patient receiving ERT. Each patient received his 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.

SD-101 for EB

We are also in Phase 3 development of a novel, late-stage, proprietary topical cream, SD-101, a potentially first-to-market therapy for the treatment of skin blistering and lesions associated with all major types of EB. ESSENCE, a Phase 3 registration-directed study, was initiated in March of 2015. ESSENCE is a randomized, double-blind, placebo-controlled study being conducted at multiple sites worldwide that is designed to evaluate the safety and efficacy of SD-101 6% in up to 150 patients with the three major types of EB, who are at least one-month old. Participants are being randomized 1:1 to two treatment groups receiving either SD-101 6% or placebo applied over their entire body once daily for three months.

The primary efficacy endpoint will be evaluation of closure of a selected target wound. In addition, time to target wound closure, changes in full-body wound, lesion coverage, and patient/caregiver reported itching and pain will be assessed. Investigators will also assess safety. An open-label extension trial, SD-006, which will evaluate long-term safety, will be offered to patients completing ESSENCE.

SD-101 for EB: Regulatory Pathway

SD-101 was one of the first therapies to receive Breakthrough Therapy designation by the FDA in 2013, following the completion of the Phase 2a initial human proof-of-concept study. The FDA and EMA each have also reviewed the Phase 2b study results and are aligned on the design of the current Phase 3 study and the global regulatory pathway forward for SD-101 based on a single Phase 3 registration-directed study. The FDA agreed to a rolling NDA in the U.S., which was initiated in the fourth quarter of 2015. Following the Phase 2b study, our Paediatric Committee of the EMA has issued a positive opinion on our Paediatric Investigation Plan (PIP) for SD-101. A PIP is part of the EMA approval process and must be accepted prior to a submission of an MAA in the EU. Results from the Phase 3 study are anticipated in the second half of

2016 to support marketing applications for SD-101 in the U.S., EU, and other regions.

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| Novel ERT for Pompe Disease |
| We are leveraging our biologics capabilities and CHART platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered rhGAA enzyme (designated ATB200) with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone to improve activity and stability. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma, Inc. (Callidus). |
| In the fourth quarter of 2015, we initiated the Phase 1/2 clinical study ATB200-02 to investigate our novel Pompe treatment paradigm in Pompe patients. The key features of this Phase 1/2 study include: |
| Open-label, dose-escalation to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of intravenous ATB200 co-administered with oral AT2221; |
| • Subjects in the first cohorts will be adult Pompe disease patients switched from currently marketed ERT; |
| Primary treatment period will be 18 weeks, with all patients eligible to enroll in an open-label extension study; and |
| • Interim data from this study are anticipated in 2016. |
| Acquisitions |
| Scioderm, Inc. |
| In September 2015, we acquired Scioderm, Inc., (Scioderm), which strengthens our pipeline significantly with the addition of a novel, late-stage, proprietary topical cream and potential first-to-market therapy for EB (SD-101). This investigational product was granted FDA breakthrough therapy designation in 2013, based on results from Phase 2 studies for the treatment of lesions in patients suffering with EB. SD-101 is currently being investigated in a Phase 3 study to support global regulatory submissions and was the first-ever treatment in EB clinical studies to show improvements in wound closure across all major EB subtypes. |

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

Callidus Biopharma, Inc.

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

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

Critical Accounting Policies, Significant Judgments and Estimates and Business Combinations

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

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There were no significant changes during the quarter ended March 31, 2016 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, 2015. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Research and Development Expenses

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

- internal costs associated with our research and clinical development activities;
- payments we make to third party contract research organizations, contract manufacturers, investigative sites, and consultants:
- technology license costs;
- manufacturing development costs;
- personnel-related expenses, including salaries, benefits, travel, and related costs for the personnel involved in drug discovery and development;
- activities relating to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and
- facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies.

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

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

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):

| | Three Months ended Ma | | |
|---|-----------------------|----|--------|
| Projects | 2016 | | 2015 |
| Third party direct project expenses | | | |
| | | | |
| Monotherapy Studies | | | |
| Migalastat (Fabry Disease Phase 3) | \$ 3,947 | \$ | 4,613 |
| SD-101 (EB-Epidermolysis Bullosa Phase 3) | 1,615 | | |
| Combination Studies | | | |
| ATB200 + AT2221 (Pompe Disease Phase 2) | 5,790 | | 3,590 |
| Fabry CHART (Fabry Disease Preclinical) | 154 | | 103 |
| Neurodegenerative Diseases (Preclinical) | | | 1 |
| Total third party direct project expenses | \$ 11,506 | \$ | 8,307 |
| Other project costs (1) | | | |
| Personnel costs | \$ 8,418 | \$ | 5,570 |
| Other costs (2) | 3,501 | | 2,236 |
| Total other project costs | \$ 11,919 | \$ | 7,806 |
| Total research and development costs | \$ 23,425 | \$ | 16,113 |

⁽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 M Ended Ma | |
|------------------------------------|---------------------|------------|
| | 2016 | 2015 |
| Expected stock price volatility | 81.2% | 77.1% |
| Risk free interest rate | 1.8% | 1.7% |
| Expected life of options (years) | 6.25 | 6.25 |
| Expected annual dividend per share | \$ 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. There have not been any RSUs awarded in 2016. The RSUs are generally subject to graded vesting and are contingent on an employee s continued service on such date. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. We expense the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

Warrants

In October 2015, the we entered into a Note and Warrant Purchase Agreement (the October 2015 Purchase Agreement) with Redmile Capital Fund, LP and certain of its affiliates (Redmile), whereby the Company sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of unsecured promissory notes and (b) 1,349,998 warrants that have a term of five years. The notes and warrants were immediately separable and issued separately. We received the proceeds related to the arrangement of \$50.0 million cash on September 28, 2015. The fair

value of the warrants were initially measured at \$8.8 million using the Black-Scholes valuation model.

On February 19, 2016, we entered into another Note and Warrant Purchase Agreement (the February 2016 Purchase Agreement) with Redmile Group, LLC and certain funds and accounts managed or advised by it, whereby we sold, on a private placement basis, (a) \$50,000,000 aggregate principal amount of unsecured promissory notes and (b) five-year warrants to purchase up to 37 shares of our common stock for every \$1,000 of the principal amount of notes purchased by each purchaser, for an aggregate of up to 1,850,000 shares of common stock issuable under the warrants. We agreed with Redmile that in full consideration of the purchase price for the notes issued under the October 2015 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purahase Agreement and we will pay Redmile any unpaid interest accrued thereunder.

Nonqualified Cash Deferral Plan

In July 2014, our Board of Directors approved the Cash Deferral Plan (the Deferral Plan), which provides certain key employees and other service providers as selected by the Compensation Committee, with an opportunity to defer the receipt of such participant s base salary, bonus and director s fees, as applicable. The Deferral Plan is intended to be a nonqualified deferred compensation plan that complies with the provisions of Section 409A of the Internal Revenue Code of 1986, as amended.

The 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.

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|---|----|----|----|-----|--------|
| | | | | | |

Results of Operations

Three Months Ended March 31, 2016 Compared to Three Months Ended March 31, 2015

Research and Development Expense. Research and development expense was \$23.4 million during the three months ended March 31, 2016, representing an increase of \$7.3 million or 45.3% from \$16.1 million for the three months ended March 31, 2015. The increase in research and development costs was primarily due to increases in contract manufacturing and clinical research costs. Contract manufacturing increased by \$2.4 million and clinical research by \$1.0 million due to scale up of Pompe ERT manufacturing and the continual progress of our programs through the clinical development process. Other increases were in personnel costs of \$2.8 million and external program support of \$1.1 million.

General and Administrative Expense. General and administrative expense was \$15.7 million for the three months ended March 31, 2016, representing an increase of \$9.3 million or 145.3% from \$6.4 million for the three months ended March 31, 2015. The increase was due to personnel costs of \$4.9 million and consulting fees of \$2.4 million. Also included within the overall increase was \$4.2 million related to pre-commercial organization costs.

Changes in Fair Value of Contingent Consideration Payable. For the three months ended March 31, 2016, we recorded expense of \$3.2 million representing an increase of \$2.2 million from the \$1.0 million of expense for the three months ended March 31, 2015. The change in the fair value resulted primarily from an increase in the Scioderm contingent consideration of \$2.2 million. The fair value is impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods and rates.

Restructuring Charges. Restructuring charges arose from the corporate restructuring implemented in the fourth quarter of 2013. This measure was intended to reduce costs and to align our resources with our key strategic priorities. The increase to the restructuring expense was \$50 thousand for three months ended March 31, 2016 as compared to \$10 thousand for the three months ended March 31, 2015, and was due to the change in fair value of the future minimum lease payments.

Depreciation expense. Depreciation expense was \$0.7 million for the three months ended March 31, 2016, representing an increase of \$0.2 million as compared to \$0.5 million for the three months ended March 31, 2015. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2016.

Interest Income. Interest income was \$0.3 million for the three months ended March 31, 2016, representing an increase of \$0.1 million from \$0.2 million for the three months ended March 31, 2015. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

Interest Expense. Interest expense was approximately \$0.9 million for three months ended March 31, 2016, representing an increase of \$0.5 million from \$0.4 million for the three months ended March 31, 2015. Interest expense was higher due to the \$50 million notes payable secured in October 2015 and the related revised agreement in February 2016, partially offset by the early retirement of the \$15 million secured loan in June 2015.

Other Income/Expense. Other expenses for the three months ended March 31, 2016 was \$52 thousand, as compared to other expenses of \$29 thousand for the three months ended March 31, 2015. The changes were from fair value changes to success fee payable related to the \$15 million loan which was paid in full during the second quarter of 2015, fair value changes to deferred compensation assets and gain/losses on foreign exchange transactions.

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Liquidity and Capital Resources

Source of Liquidity

On February 26, 2016, we entered into the Sales Agreement (the Sales Agreement) with Cowen and Company, LLC (Cowan) to create an at-the-market equity program under which the Company from time to time may offer and sell shares of its common stock, par value \$0.01 per share, having an aggregate offering price of up to \$100 million through Cowen (the ATM Facility). Sales of the ATM Facility shares under the Sales Agreement may be made in transactions that are deemed to be at the market offerings as defined in Rule 415 promulgated under the Securities Act of 1933, as amended, including sales made directly on The NASDAQ Global Market, on any other existing trading market for the common stock or through a market maker. In addition, with our prior written approval, Cowen may also sell shares of common stock by any other method permitted by law, including in negotiated transactions. Cowen will act as sales agent using its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of The NASDAQ Stock Market LLC. There is no arrangement for funds to be received in an escrow, trust or similar arrangement. Cowen will be entitled to compensation at a fixed commission rate up to 3.0% of the gross proceeds per share sold through it as sales agent under the Sales Agreement. In April 2016, we sold 2.1 million shares of common stock under the ATM Sales Agreement resulting in net proceeds of \$16.2 million, which included Cowen s commission of \$0.5 million.

In October 2015, we entered into the October 2015 Purchase Agreement with Redmile, who owned approximately 6.7% of our common stock as of December 31, 2015, whereby we sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of unsecured promissory notes and (b) 1,349,998 warrants that have a term of five years. We received the proceeds related to the arrangement of \$50.0 million cash on September 28, 2015. The payment terms under the purchase agreement contained two installments, the first \$15.0 million in October 2017 and the balance \$35.0 million in October 2020. Interest was payable at 4.1% on a monthly basis over the term of the loan. On February 19, 2016, we entered into the February 2016 Purchase Agreement with Redmile , whereby we sold, on a private placement basis, (a) \$75.0 million aggregate principal amount of unsecured promissory notes of which \$50.0 million becomes available immediately and the balance \$25.0 million becomes available subject to certain conditions and (b) 1.9 million warrants that have a term of five-years. The payment terms under the February 2016 Purchase Agreement contains two installments, the first \$15.0 million is due in October 2017 and the balance \$35.0 million is due in October 2021. For each tranche, interest will accrue at 3.875% but will go unpaid until final maturity. We agreed with Redmile that in full consideration of the purchase price for the notes issued under the October 2015 Purchase Agreement, Redmile surrendered for cancellation all notes and warrants acquired from the October 2015 Purchase Agreement and we paid Redmile the interest accrued thereunder.

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.

As of March 31, 2016, we had cash and cash equivalents and marketable securities of \$165.9 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 operations for the three months ended March 31, 2016 was \$44.9 million, due primarily to the net loss for the three months ended March 31, 2016 of \$43.7 million and the change in operating assets and liabilities of \$9.9 million. The change in operating assets and liabilities was primarily due to a decrease in accounts payable and accrued expenses of \$9.7 million and increase in deferred investment balances of \$0.4 million.

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

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Net Cash (Used in)/ Provided by Investing Activities

Net cash used in investing activities for the three months ended March 31, 2016 was \$0.4 million and reflects \$50.7 million for the purchase of marketable securities, \$2.9 million for the acquisition of property and equipment, partially offset by \$53.2 million for the sale and redemption of marketable securities.

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

Net Cash (Used in)/ Provided by Financing Activities

Net cash used in financing activities for the three months ended March 31, 2016 was \$0.5 million, which reflects \$0.7 million from vesting of RSUs, partially offset by \$0.2 million received from exercise of stock options.

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

Funding Requirements

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

- the progress and results of our clinical trials of our drug candidates, including migalastat HCl;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage diseases;
- the future results of ongoing or later clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and market acceptance of SD-101;

- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products or technologies;
- our ability to successfully incorporate Scioderm and its products and technology into our business, including the possibility that the expected benefits of the transaction will not be fully realized by us or may take longer to realize than expected; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

We do not anticipate that we will generate revenue from commercial sales until second quarter of 2016 at the earliest, 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

We acquired exclusive worldwide patent rights to develop and commercialize migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with Mt. Sinai School of Medicine (MSSM). This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2019, subject to any patent term extension that may be granted, or 2024 we develop a product for combination therapy (pharmacological chaperone plus ERT) and a patent issues from the pending application covering combination therapy, subject to any patent term extension that may be granted. Under this agreement, to date we have paid no upfront or annual license fees and has no milestone or future payments other than royalties on net sales.

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

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

As part of the merger agreement with Scioderm, we have agreed to pay up to an additional \$361 million to Scioderm shareholders, option holders, and warrant holders upon achievement of certain clinical and regulatory milestones, and \$257 million to Scioderm shareholders, option holders, and warrant holders upon achievement of certain sales milestones. If SD-101 is approved, EB qualifies as a rare pediatric disease and we will request a Priority Review Voucher. If the Priority Review Voucher is obtained and subsequently sold, we will pay Scioderm shareholders, option holders and warrant holders the lesser of \$100 million in the aggregate or 50% of the proceeds of such sale.

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

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

Recent Accounting Pronouncements

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

ITEM 3. Quantitative and Qualitative Disclosures about Market Risk

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and

marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At March 31, 2016, we held \$165.9 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. At March 31, 2016, our cash, cash equivalents and available for sale securities were all due on demand or within one year. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

We have operated primarily in the U.S. with international operations increasing since the last quarter of 2015. We do conduct some clinical activities with vendors outside the U.S. While most expenses are paid in U.S. dollars, 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.

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ITEM 4. CONTROLS AND PROCEDURES

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

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

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for the District of New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company s common stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. The Company anticipates that these lawsuits will be consolidated into a single action.

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

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

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

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

ITEM 1A. RISK FACTORS

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

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ITEM 4. MINE SAFETY DISCLOSURES

| ITEM 2. UNREGISTERED SALES (| OF EQUITY SECURIT | TES AND USE OF I | PROCEEDS | |
|--|---|---|--|---|
| Recent Sales of Unregistered Securities | es | | | |
| None. | | | | |
| Issuer Purchases of Equity Securities | | | | |
| The following table sets forth purchases | of our common stock fo | or the three months en | nded March 31, 2016: | |
| | | (b) | (c) Total number of shares purchased as | (d) Maximum number of shares |
| Period | (a) Total number of shares purchased | Average Price Paid per Share | part of publicly announced plans or programs | that may yet be purchased under the plans or programs |
| January 1, 2016 to February 29, 2016 | 87,361 \$ | 8.73 | programs | 152,639 |
| Pursuant to a restricted stock award data RSU. Some of the RSUs that vested in 2 July 2016, subject generally to the empl tax withholding rate of 25%, 2.22% for | ed April 10, 2014 betwee 2015 were released in the oyee s continued emplo | en Amicus and certair three months ended syment with the Comp | n employee recipients, co March 31, 2016. The rep pany. In order to comply | mainder of the RSUs will vest in with the minimum statutory federal |
| employee surrendered to us a portion of then vested. | | | | |
| ITEM 3. DEFAULTS UPON SENIOR | R SECURITIES | | | |

Not applicable. ITEM 5. OTHER INFORMATION None.

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ITEM 6. EXHIBITS

| Exhibit Number | Description |
|-------------------|--|
| 3.1(1) | Restated Certificate of Incorporation |
| 3.2(2) | Certificate of Amendment to the Company s Restated Certificate of Incorporation, as amended. |
| 3.3 (3) | Amended and Restated By-laws |
| 10.1 (4) | Sales Agreement, dated February 26, 2016, by and between Amicus Therapeutics, Inc. and Cowen and Company, LLC |
| 10.2 (5) | Retention Bonus Letter, dated March 10, 2016, by and between Amicus Therapeutics, Inc. and Jay Barth, M.D. |
| 10.3 (6) | Note and Warrant Purchase Agreement by and among Amicus Therapeutics, Inc. and the purchasers identified on the signature pages thereto, dated February 19, 2016. |
| 31.1 | Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 31.2 | Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 32.1 | Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 101 | The following financial information from this Quarterly Report on Form 10-Q for the three months ended March 31, 2016, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements. |

- (1) Incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on August 5, 2015.
- (2) Incorporated by reference to Exhibit 3.2 to our Quarterly Report on Form 10-Q filed on August 5, 2015.
- (3) Incorporated by reference to Exhibit 3.3 to our Registration Statement on Form S-1.
- (4) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on February 26, 2016.
- (5) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on March 15, 2016.
- (6) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on February 22, 2016.

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SIGNATURES

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

AMICUS THERAPEUTICS, INC.

Date: May 3, 2016 By: /s/ John F. Crowley

John F. Crowley

Chairman and Chief Executive Officer (Principal Executive Officer)

Date: May 3, 2016 By: /s/ William D. Baird III

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

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| Exhibit Number | Description |
|-------------------|--|
| 31.1 | Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 31.2 | Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 32.1 | Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 101 | The following financial information from this Quarterly Report on Form 10-Q for the three months ended March 31, 2016, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements. |
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