

AMICUS THERAPEUTICS INC

Form 10-Q

November 04, 2015

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

Washington, D.C. 20549

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 2015

OR

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

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

Commission file number 001-33497

## Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

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

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

**1 Cedar Brook Drive, Cranbury, NJ 08512**

(Address of Principal Executive Offices and Zip Code)

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

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

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

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

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller Reporting Company

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

The number of shares outstanding of the registrant's common stock, \$.01 par value per share, as of October 29, 2015 was 124,899,439 shares.



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AMICUS THERAPEUTICS, INC.

Form 10-O for the Quarterly Period Ended September 30, 2015

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

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**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

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

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

- the progress and results of our clinical trials of our drug candidates, including our pharmacological chaperone migalastat HCl ( Galafold );
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new Fabry enzyme replacement therapy ( ERT ) cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage disorders ( LSDs );
- the future results of on-going or later clinical trials for SD-101 ( Zorblisa ), including our ability to obtain regulatory approvals and commercialize Zorblisa and market acceptance of Zorblisa;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to obtain reimbursement for Galafold;
- our ability to commercialize Galafold in the European Union;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property-related claims;
- the extent to which we acquire or invest in businesses, products and technologies;

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- 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, 2014 and on Form 10-Q for the period ended June 30, 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 documents that we reference herein. We do not assume any obligation to update any forward-looking statements.

Table of Contents**PART I. FINANCIAL INFORMATION****Item 1. Financial Statements (unaudited)****Amicus Therapeutics, Inc.****Consolidated Balance Sheets***(Unaudited)***(in thousands, except share and per share amounts)**

|  | September 30,<br>2015 | December 31,<br>2014 |
|--|-----------------------|----------------------|
| <b>Assets:</b>   |                       |                      |
| Current assets:  |                       |                      |
| Cash and cash equivalents  | \$ 19,439             | \$ 24,074            |
| Investments in marketable securities   | 217,070               | 127,601              |
| Prepaid expenses and other current assets  | 3,544                 | 2,902                |
| Total current assets   | 240,053               | 154,577              |
| Investments in marketable securities   | 15,428                | 17,464               |
| Property and equipment, less accumulated depreciation of \$12,776 and \$11,520 at September 30, 2015 and December 31, 2014, respectively   | 3,855                 | 2,811                |
| In-process research & development  | 518,810               | 23,000               |
| Goodwill   | 207,564               | 11,613               |
| Other non-current assets   | 982                   | 502                  |
| <b>Total Assets</b>  | <b>\$ 986,692</b>     | <b>\$ 209,967</b>    |
| <b>Liabilities and Stockholders' Equity</b>  |                       |                      |
| Current liabilities:   |                       |                      |
| Accounts payable and accrued expenses  | \$ 30,565             | \$ 16,345            |
| Current portion of contingent consideration payable  | 5,300                 |                      |
| Current portion of secured loan  |                       | 3,840                |
| Total current liabilities  | 35,865                | 20,185               |
| Deferred reimbursements  | 36,620                | 36,620               |
| Secured loan, less current portion   |                       | 10,510               |
| Due to related party   | 50,000                |                      |
| Contingent consideration payable, less current portion   | 277,684               | 10,700               |
| Deferred tax liability   | 207,213               | 9,186                |
| Other non-current liabilities  | 555                   | 588                  |
| Commitments and contingencies  |                       |                      |
| Stockholders' equity:  |                       |                      |
| Common stock, \$.01 par value, 250,000,000 shares authorized, 124,617,490 shares issued and outstanding at September 30, 2015, 95,556,277 shares issued and outstanding at December 31, 2014 | 1,304                 | 1,015                |



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|   |                   |                   |
|---|-------------------|-------------------|
| Additional paid-in capital                        | 914,263           | 568,743           |
| Accumulated other comprehensive loss              | (142)             | (132)             |
| Accumulated deficit                               | (536,670)         | (447,448)         |
| Total stockholders' equity                        | 378,755           | 122,178           |
| <b>Total Liabilities and Stockholders' Equity</b> | <b>\$ 986,692</b> | <b>\$ 209,967</b> |

*See accompanying notes to consolidated financial statements*

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

|  | Three Months<br>Ended September 30, |             | Nine Months<br>Ended September 30, |             |
|--|-------------------------------------|-------------|------------------------------------|-------------|
|  | 2015                                | 2014        | 2015                               | 2014        |
| <b>Revenue:</b>  |                                     |             |                                    |             |
| Research revenue   |                                     | \$ 293      |                                    | \$ 1,224    |
| Total revenue  |                                     | 293         |                                    | 1,224       |
| <b>Operating Expenses:</b>                                   |                                     |             |                                    |             |
| Research and development                                     | \$ 20,971                           | \$ 12,049   | \$ 54,318                          | \$ 32,019   |
| General and administrative                                   | 15,372                              | 5,270       | 30,077                             | 15,199      |
| Changes in fair value of contingent consideration payable    | 1,300                               | (600)       | 2,400                              | (400)       |
| Restructuring charges  | 7                                   | 15          | 44                                 | (74)        |
| Loss on extinguishment of debt                               |                                     |             | 952                                |             |
| Depreciation   | 395                                 | 375         | 1,256                              | 1,183       |
| Total operating expenses                                     | 38,045                              | 17,109      | 89,047                             | 47,927      |
| Loss from operations   | (38,045)                            | (16,816)    | (89,047)                           | (46,703)    |
| <b>Other income (expenses):</b>                              |                                     |             |                                    |             |
| Interest income  | 316                                 | 55          | 645                                | 133         |
| Interest expense   | (17)                                | (377)       | (727)                              | (1,106)     |
| Other expense  | (54)                                | (11)        | (93)                               | (30)        |
| Net loss   | \$ (37,800)                         | \$ (17,149) | \$ (89,222)                        | \$ (47,706) |
| Net loss per common shares basic and diluted                 | \$ (0.32)                           | \$ (0.22)   | \$ (0.85)                          | \$ (0.68)   |
| Weighted-average common shares outstanding basic and diluted | 118,724,882                         | 78,889,346  | 104,885,956                        | 70,216,251  |

*See accompanying notes to consolidated financial statements*

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

|   | <b>Three Months<br/>Ended September 30,</b> |             | <b>Nine Months<br/>Ended September 30,</b> |             |
|---|---|-------------|--|-------------|
|   | <b>2015</b>                                 | <b>2014</b> | <b>2015</b>                                | <b>2014</b> |
| Net loss  | \$ (37,800)                                 | \$ (17,149) | \$ (89,222)                                | \$ (47,706) |
| Other comprehensive income/(loss):  |   |             |  |             |
| Unrealized (loss)/gain on available-for-sale securities                           | (91)  | 4           | (10)                                       | 1           |
| Other comprehensive (loss)/gain before income taxes                               | (91)  | 4           | (10)                                       | 1           |
| Provision for income taxes related to other comprehensive (loss)/income items (a) |   |             |  |             |
| Other comprehensive (loss)/income   | \$ (91)                                     | \$ 4        | \$ (10)                                    | \$ 1        |
| Comprehensive loss  | \$ (37,891)                                 | \$ (17,145) | \$ (89,232)                                | \$ (47,705) |

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

*See accompanying notes to consolidated financial statements*

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

|   | Nine Months<br>Ended September 30 |             |
|---|-----------------------------------|-------------|
|   | 2015                              | 2014        |
| <b>Operating activities</b>   |                                   |             |
| Net loss  | \$ (89,222)                       | \$ (47,706) |
| Adjustments to reconcile net loss to net cash used in operating activities: |                                   |             |
| Non-cash interest expense   | 136                               | 176         |
| Depreciation  | 1,256                             | 1,183       |
| Stock-based compensation  | 6,929                             | 4,398       |
| Restructuring charges   | 44                                | (74)        |
| Loss on extinguishment of debt  | 952                               |             |
| Non-cash changes in the fair value of contingent consideration payable      | 2,400                             | (400)       |
| Changes in operating assets and liabilities:                                |                                   |             |
| Receivable due from collaboration agreements                                |                                   | 790         |
| Prepaid expenses and other current assets                                   | (668)                             | 3,433       |
| Other non-current assets  | (540)                             | 26          |
| Accounts payable and accrued expenses                                       | 14,118                            | 1,213       |
| Non-current liabilities   | (33)                              | (200)       |
| Net cash used in operating activities                                       | (64,628)                          | (37,161)    |
| <b>Investing activities</b>   |                                   |             |
| Sale and redemption of marketable securities                                | 133,418                           | 47,959      |
| Purchases of marketable securities  | (220,861)                         | (75,109)    |
| Purchases of property and equipment   | (2,246)                           | (192)       |
| Acquisitions, net cash of acquired  | (141,060)                         |             |
| Net cash (used in) investing activities                                     | (230,749)                         | (27,342)    |
| <b>Financing activities</b>   |                                   |             |
| Proceeds from issuance of common stock, net of issuance costs               | 243,042                           | 38,736      |
| Payments of secured loan agreement  | (15,291)                          | (299)       |
| Proceeds from related party   | 50,000                            |             |
| Purchase of vested restricted stock units                                   | (1,682)                           |             |
| Proceeds from exercise of stock options                                     | 10,673                            | 2,097       |
| Proceeds from exercise of warrants  | 4,000                             |             |
| Net cash provided by financing activities                                   | 290,742                           | 40,534      |
| Net decrease in cash and cash equivalents                                   | (4,635)                           | (23,969)    |
| Cash and cash equivalents at beginning of period                            | 24,074                            | 43,640      |
| Cash and cash equivalents at end of period                                  | \$ 19,439                         | \$ 19,671   |
| <b>Supplemental disclosures of cash flow information</b>                    |                                   |             |
| Cash paid during the period for interest                                    | \$ 605                            | \$ 864      |

*See accompanying notes to consolidated financial statements*



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**Amicus Therapeutics, Inc.**

**Notes to Consolidated Financial Statements**

**Note 1. Description of Business**

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

Amicus Therapeutics, Inc. (the Company, we, us, or our) was incorporated on February 4, 2002 in Delaware and is a biopharmaceutical company focused on the discovery, development and commercialization of 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 (Zorblisa), 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.

Our Fabry franchise strategy is to develop migalastat HCl for all patients with Fabry disease - as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients. The Company has submitted a marketing application for migalastat monotherapy (Galafold) in the European Union, and plans to continue working with the U.S. Food and Drug Administration (FDA) to determine the optimal U.S. registration pathway.

In September 2015, Amicus acquired Scioderm, Inc., (Scioderm), which strengthens the Company's pipeline 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. Zorblisa 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.

The Company 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. 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 Zorblisa is approved, EB qualifies as a rare pediatric disease and Amicus will request a Priority Review Voucher. If the Priority Review Voucher 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.

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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 expects to provide an update on the U.S. regulatory plans in the first half of 2016.

In June 2015, the European Medicines Agency ( EMA ) validated the Company 's Marketing Authorization Application ( MAA ) submission for Galafold and the Centralized Procedure has begun under Accelerated Assessment. The Committee for Medicinal Products for Human Use ( CHMP ) may shorten the MAA review period from 210 days, under standard review, to 150 days under Accelerated Assessment. The CHMP opinion is then reviewed by the European Commission, which generally issues a final decision on European Union ( EU ) approval within three months. The MAA submission will be reviewed in the Centralized Procedure, which if authorized, provides a marketing license valid in all 28 EU member states. Once authorized, the Company would then begin the country-by-country reimbursement approval process. Following the MAA validation, the Company is also initiating the regulatory submission process in several additional geographies.

In October 2015, the Company entered into a Note and Warrant Purchase Agreement (the 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

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principal amount of its unsecured promissory notes ( Notes ) and (b) five-year warrants ( Warrants ) for 1,349,998 shares of Common Stock.

For more details, refer to Note 14. Subsequent Events

In June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share, with net proceeds of \$243.0 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

The Company had an accumulated deficit of approximately \$536.7 million at September 30, 2015 and anticipates incurring losses through the fiscal year ending December 31, 2015 and beyond. The Company has not yet generated commercial sales revenue and has been able to fund its operating losses to date through the sale of its redeemable convertible preferred stock, issuance of convertible notes, net proceeds from its initial public offering and subsequent stock offerings, collaboration payments from partners and other financing arrangements. The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2017.

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

***Basis of Presentation***

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

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2014. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K for the fiscal year ended December 31, 2014.

***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 nine months ended September 30, 2015, as compared to the significant accounting policies disclosed in Note 2 of the Consolidated Financial Statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2014. However, the following accounting policies are the most critical in fully understanding and evaluating the Company's financial condition and results of operations.

***Revenue Recognition***

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

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

The Company's current revenue recognition policies provide that, when a collaboration arrangement contains multiple deliverables, such as license and research and development services, the Company allocates revenue to each separate unit of accounting based on a selling price hierarchy. The selling price hierarchy for a deliverable is based on (i) its vendor specific

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objective evidence ( VSOE ) if available, (ii) third party evidence ( TPE ) if VSOE is not available, or (iii) best estimated selling price ( BESP ) if neither VSOE nor TPE is available. The Company would establish the VSOE of selling price using the price charged for a deliverable when sold separately. The TPE of selling price would be established by evaluating largely similar and interchangeable competitor products or services in standalone sales to similarly situated customers. The BESP would be established considering internal factors such as an internal pricing analysis or an income approach using a discounted cash flow model.

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

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

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

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

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

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

***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.

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***Contingent Liabilities***

On an ongoing basis, the Company may be involved in various claims, and legal proceedings. On a quarterly basis, the Company reviews the status of each significant matter and assesses its potential financial exposure. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount can be reasonably estimated, the Company will accrue a liability for the estimated loss. Because of uncertainties related to claims and litigation, accruals will be based on 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 September 2015, the FASB issued Accounting Standards Update (ASU) 2015-16 Business Combinations (Topic 805): *Simplifying the Accounting for Measurement-Period Adjustments*. The amendments in ASU 2015-16 require that an acquirer recognize adjustments to estimated amounts that are identified during the measurement period in the reporting period in which the adjustment amounts are determined. The amendments require that the acquirer record, in the same period's financial statements, the effect on earnings of changes in depreciation, amortization, or other income effects, if any, as a result of the change to the estimated amounts, calculated as if the accounting had been completed at the acquisition date. The amendments also require an entity to present separately on the face of the income statement or disclose in the notes the portion of the amount recorded in current-period earnings by line item that would have been recorded in previous reporting periods if the adjustment to the estimated amounts had been recognized as of the acquisition date. The ASU is effective for public business entities for fiscal years beginning after December 15, 2015, including interim periods within those fiscal years. Early adoption is permitted for financial statements that have not been issued. We have early adopted this standard as of September 30, 2015 and this standard had no impact on our consolidated financial statements.

In April 2015, the FASB issued ASU 2015-05, Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): *Customer's Accounting for Fees Paid in a Cloud Computing Arrangement*. The amendments in ASU 2015-05 provide guidance to customers about whether a cloud computing arrangement includes a software license. If a cloud computing arrangement includes a software license, then the customer should account for the software license element of the arrangement consistent with the acquisition of other software licenses. If a cloud computing arrangement does not include a software license, the customer should account for the arrangement as a service contract. The amendments do not change the accounting for a customer's accounting for service contracts. As a result of the amendments, all software licenses within the scope of Subtopic 350-40 will be accounted for consistent with other licenses of intangible assets. The ASU is effective for financial statements issued for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. Early adoption is permitted. We are currently assessing the impact that this standard will have on our consolidated financial statements.

In April 2015, the FASB issued ASU 2015-03, *Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs*. The amendments in ASU 2015-03 are intended to simplify the presentation of debt issuance costs. These amendments require that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. The recognition and measurement guidance for debt issuance costs are not affected by the amendments in this ASU. The ASU is effective for financial statements issued for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. The guidance in ASU 2015-03 (see paragraph 835-30-45-1A) does not address presentation or subsequent measurement of debt issuance costs related to line-of-credit arrangements. Given the absence of authoritative guidance within ASU 2015-03 for debt issuance costs related to line-of-credit arrangements, the SEC staff stated in June 2015 that they would not object to an entity deferring and presenting debt issuance costs as an asset and subsequently amortizing the deferred debt issuance costs ratably over the term of the line-of-credit arrangement, regardless of whether there are any outstanding borrowings on the line-of-credit arrangement. ASU 2015-15 *Imputation of*

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*Interest* adds the SEC paragraph to the Topic. We are currently assessing the impact that this standard will have on our consolidated financial statements.

In November 2014, the FASB issued ASU 2014-17, *Business Combinations (Topic 805): Pushdown Accounting*. The amendments in ASU 2014-17 provide an acquired entity with an option to apply pushdown accounting in its separate financial statements upon occurrence of an event in which an acquirer obtains control of the acquired entity. The ASU is effective on November 18, 2014. After the effective date, an acquired entity can make an election to apply the guidance to future change-in-control events or to its most recent change-in-control event. However, if the financial statements for the period in which the most recent change-in-control event occurred already have been issued or made available to be issued, the application of this guidance would be a change in accounting principle. The adoption of this guidance did not have a significant impact on our consolidated financial statements.

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

In May 2014, FASB issued ASU 2014-09, *Revenue From Contracts With Customers*, that outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The ASU is based on the principle that an entity should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to fulfill a contract. Entities have the option of using either a full retrospective or a modified retrospective approach for the adoption of the new standard. In July 2015, the Financial Accounting Standards Board voted to delay the effective date of this standard until the first quarter of 2018. Companies are permitted to early adopt the standard in the first quarter of 2017. Presently, we are assessing the effect the adoption of ASU 2014-09 will have on our consolidated financial statements.

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

As of September 30, 2015, the Company held \$19.4 million in cash and cash equivalents and \$232.5 million of available-for-sale securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income/(loss) in the statements of comprehensive loss. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge. To date, only temporary impairment adjustments have been recorded.

Consistent with the Company's investment policy, the Company does not use derivative financial instruments in its investment portfolio. The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating. Investments that have original maturities or greater than 3 months but less than 1 year are classified as short-term and investments with maturities that are greater than 1 year are classified as long-term.

Cash and available-for-sale securities are all current unless mentioned otherwise and consisted of the following as of September 30, 2015 and December 31, 2014 (in thousands):

|                                       | As of September 30, 2015 |                 |                 |            |
|---------------------------------------|--------------------------|-----------------|-----------------|------------|
|                                       | Cost                     | Unrealized Gain | Unrealized Loss | Fair Value |
| Cash balances                         | \$ 19,439                | \$              | \$              | \$ 19,439  |
| Corporate debt securities             | 127,841                  | 19              | (210)           | 127,650    |
| Commercial paper                      | 104,450                  | 48              |                 | 104,498    |
| Certificate of deposit                | 350                      |                 |                 | 350        |
|                                       | \$ 252,080               | \$ 67           | \$ (210)        | \$ 251,937 |
| Included in cash and cash equivalents | \$ 19,439                | \$              | \$              | \$ 19,439  |
| Included in marketable securities     | 232,641                  | 67              | (210)           | 232,498    |
| Total cash and marketable securities  | \$ 252,080               | \$ 67           | \$ (210)        | \$ 251,937 |

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

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Unrealized gains and losses are reported as a component of other comprehensive income/ (loss) in the statements of comprehensive loss. For the nine months ended September 30, 2015, unrealized holding loss of \$10 thousand and for the year ended December 31, 2014, unrealized holding loss of \$132 thousand, were included in the statement of comprehensive loss.



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For the nine months ended September 30, 2015 and the year ended December 31, 2014, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of September 30, 2015 and December 31, 2014 reflect temporary impairments that have not been recognized and have been in a loss position for less than twelve months. The fair value of these available for sale securities in unrealized loss positions was \$103.3 million and \$129.2 million as of September 30, 2015 and December 31, 2014, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income ( AOCI ) in the statements of comprehensive loss. The changes in AOCI associated with the unrealized holding gain on available-for-sale investments during the three and nine months, ended September 30, 2015 and 2014, were as follows (in thousands):

|   | Three Months Ended<br>September 30, |        | Nine Months Ended<br>September 30, |      |
|---|-------------------------------------|--------|------------------------------------|------|
|   | 2015                                | 2014   | 2015                               | 2014 |
| Balance, beginning                        | \$ (51)                             | \$ (2) | \$ (132)                           | \$ 1 |
| Current period changes in fair value, (a) | (91)                                | 4      | (10)                               | 1    |
| Reclassification of earnings, (a)         |                                     |        |                                    |      |
| Balance, ending                           | \$ (142)                            | \$ 2   | \$ (142)                           | \$ 2 |

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

**Note 4. Acquisitions****Acquisition of Scioderm, Inc.**

On September 30, 2015, Amicus acquired Scioderm, a privately-held biopharmaceutical company focused on developing innovative therapies for treating the rare disease Epidermolysis Bullosa ( EB ). The acquisition leverages the Scioderm development team's EB expertise with Amicus' global clinical infrastructure to advance Zorblisa toward regulatory approvals and Amicus' 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.

As a result, Scioderm became a wholly owned subsidiary of Amicus. At the effective date of the acquisition, Amicus paid holders of Scioderm's (i) capital stock, (ii) options to purchase Scioderm's common stock, (iii) restricted stock units and (iv) warrants to purchase Scioderm's common stock (collectively, the Effective Time Holders ), an amount equal to (i) \$220 million, plus (ii) the exercise price of all outstanding options and warrants to purchase Scioderm's common stock, plus (iii) Scioderm's cash and cash equivalents (with an adjustment to account for closing working capital and Scioderm's fees and expenses, which include employee bonuses and certain severance payments) (collectively, the Initial Amount ). \$135.6 million of the Initial Amount was paid in cash and the remaining \$79.6 million was paid in shares of Common Stock. On

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September 30, 2015, Amicus also completed its previously announced agreement with all of the holders of Scioderm's Series B Preferred Stock, par value \$0.001 per share (the Series B Additional Purchase Price Agreement), pursuant to which Amicus made payments of \$5.5 million in cash and the remaining \$3.2 million was paid in shares of Common Stock directly to the holders of Scioderm Series B Preferred Stock. Payments under the Series B Additional Purchase Price Agreement were made pro rata based on the number of shares of Scioderm Series B Preferred Stock held.

The preliminary assessment of the fair value of the contingent acquisition consideration payments on the acquisition date was \$269.9 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a range of rates between 0.4% and 1.2% as interpolated from the U.S. Treasury constant maturity yield curve over the time frame of anticipated milestones. 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.

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The allocation of the purchase price accounting is preliminary as the Company has not yet completed its analysis of estimating the fair value of the assets and liabilities acquired. The following table presents the preliminary allocation of the purchase consideration, including the contingent acquisition consideration payable, based on fair value:

|   | (in thousands) |
|---|----------------|
| Upfront cash payments   | \$ 141,060     |
| Upfront equity payments   | 82,845         |
| Contingent acquisition consideration payable                    | 269,884        |
| Total consideration   | 493,789        |
| Property, plant and equipment, net                              | 55             |
| Intangible assets In-process Research and Development ( IPR&D ) | 495,810        |
| Total identifiable assets acquired                              | 495,865        |
| Deferred tax liability  | (198,027)      |
| Total liabilities assumed                                       | (198,027)      |
| Net identifiable assets acquired                                | 297,838        |
| Goodwill  | 195,951        |
| Net assets acquired   | \$ 493,789     |

A substantial portion of the assets acquired consisted of intangible assets related to Zorblisa. The Company determined that the estimated acquisition-date fair values of the indefinite lived IPR&D related to the Zorblisa was \$495.8 million. The Company is still in the process of valuing the assets acquired and liabilities assumed; therefore the allocation of the acquisition consideration is subject to change.

The \$198.0 million of deferred tax liabilities relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes. The goodwill results from the recognition of the deferred tax liability on the intangible assets as well as synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets. None of the goodwill is expected to be deductible for income tax purposes. The Company recorded the goodwill in the consolidated balance sheet as of the acquisition date.

The Company recognized \$2.8 million of acquisition-related transaction costs in selling, general and administrative expenses during 2015, which consisted primarily of consulting and legal fees related to the acquisition.

The following unaudited consolidated pro forma financial information presents the combined results of operations of the Company and Scioderm as if the acquisition had occurred as of January 1, 2015. The unaudited pro forma consolidated financial information is not necessarily indicative of what the Company's consolidated results of operations actually would have been had the acquisition been completed as of January 1, 2015. In addition, the unaudited pro forma consolidated financial information does not attempt to project the future results of operations of the Company combined with Scioderm.

There were no revenues reported for Scioderm during the three and nine months ended September 30, 2015. There were no nonrecurring adjustments in the Pro Forma Scioderm results for the three and nine months ended September 30, 2015.

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| Unaudited Pro Forma Consolidated Information:<br>( in thousands) | Nine months ended September 30, |             |
|--|---------------------------------|-------------|
|  | 2015                            | 2014        |
| Revenue  | \$                              | \$ 1,224    |
| Net loss   | \$ (99,300)                     | \$ (54,167) |

**Acquisition of Callidus Biopharma, Inc.**

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

In consideration for the merger, the Company agreed to issue an aggregate of 7.2 million shares of its common stock, par value \$0.01 per share, to the former stockholders of Callidus. In addition, the Company will be obligated to make additional payments to the former stockholders of Callidus upon the achievement by the Company of certain clinical milestones of up to \$35 million and regulatory approval milestones of up to \$105 million as set forth in the Merger Agreement, provided that the aggregate consideration shall not exceed \$130 million. The Company may, at its election, satisfy certain milestone payments identified in the Merger Agreement aggregating \$40 million in shares of its Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Select Market for the ten (10) trading days

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immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the Merger Agreement, the rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash.

The fair value of the contingent acquisition consideration payments on the acquisition date was \$10.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a discount rate of 11.5% and various probability factors. As of September 30, 2015, the range of outcomes and assumptions used to develop these estimates has changed to better reflect the probability of certain milestone outcomes. (see Note 8. Assets and Liabilities Measured at Fair Value, for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). The Company determined the fair value of the contingent consideration to be \$13.1 million at September 30, 2015, of which \$5.3 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of \$1.3 million and \$2.4 million for three and nine months ended September 30, 2015, respectively. The expense is recorded as part of operating expense in the Consolidated Statement of Operations. For further information, see Note 5. Goodwill & Note 6. Intangible Assets.

**Note 5. Goodwill and Intangible Assets**

In connection with the acquisitions discussed in Note 4. Acquisitions, the Company has recognized goodwill of \$207.6 million. The following table represents the changes in goodwill for the nine months ended September 30, 2015:

|  | (in millions) |       |
|--|---------------|-------|
| Balance at December 31, 2014                                 | \$            | 11.6  |
| Goodwill related to the acquisition of Scioderm (See Note 4) |               | 196.0 |
| Balance at September 30, 2015                                | \$            | 207.6 |

In connection with the acquisitions discussed in Note 4. Acquisitions, the Company recognized IPR&D of \$518.8 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 nine months ended September 30, 2015 (in thousands):

|   | (in millions) |       |
|---|---------------|-------|
| Balance at December 31, 2014                              | \$            | 23.0  |
| IPR&D related to the acquisition of Scioderm (See Note 4) |               | 495.8 |
| Balance at September 30, 2015                             | \$            | 518.8 |

During the 2014 impairment assessment, it was determined that the goodwill and intangible assets had not been impaired and there were no changes to these balances in 2014. Goodwill and intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. For the nine months ended September 30, 2015, there were no indicators of impairment.



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**Note 6. Related Party Transaction**

During September 2015, the Company was in the process of negotiating a finance arrangement with Redmile Capital Fund, LP and certain of its affiliates (collectively, Redmile ). The Company received the proceeds related to the arrangement of \$50.0 million cash on September 28, 2015 and has recorded this liability on the balance sheet as Due to related party as of September 30, 2015. As discussed in Note 14. Subsequent Events the Company completed the transaction on October 1, 2015. As of September 30, 2015, Redmile beneficially owned approximately 5% of the Company's outstanding shares of common stock.

**Note 7. Stockholders' Equity**

*Common Stock and Warrants*

As of September 30, 2015, the Company was authorized to issue 250 million shares of common stock. Dividends on common stock will be paid when, and if, declared by the board of directors. Each holder of common stock is entitled to vote on all matters that are appropriate for stockholder voting and is entitled to one vote for each share held.

In September 2015, the Company acquired Scioderm through a cash and stock transaction. As part of the acquisition, the Company paid holders of Scioderm an amount equal to \$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 shares. The Company agreed to pay up to an additional \$361 million 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.

In June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share, with net proceeds of \$243.0 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In November 2014, we sold a total of 15.9 million shares of our common stock, par value \$0.01 per share, at a public offering price of \$6.50 per share. The aggregate offering proceeds were approximately \$97.2 million.

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

*Nonqualified Cash Plan*

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

Deferred compensation amounts under the Deferral Plan as of September 30, 2015 were approximately \$0.5 million, as compared to \$0.1 million on December 31, 2014 and are included in other long-term liabilities. As of December 31, 2014, the amounts deferred under the Deferral Plan had not been invested and the investments were subsequently made in the nine months ended September 30, 2015. Deferral Plan assets as of September 30, 2015 were \$0.5 million and are classified as trading securities. The Deferred Plan assets are recorded at fair value with changes in the investments' fair value recognized in the period they occur. During the three and nine months ended September 30, 2015, income from the investments was \$1 thousand and \$8 thousand respectively and unrealized loss was \$54 thousand and \$64 thousand respectively.



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

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

|                                    | Three Months ended<br>September 30, |         | Nine Months ended<br>September 30, |         |
|------------------------------------|-------------------------------------|---------|------------------------------------|---------|
|                                    | 2015                                | 2014    | 2015                               | 2014    |
| Expected stock price volatility    | 74.4%                               | 81.0%   | 75.4%                              | 81.3%   |
| Risk free interest rate            | 1.7%                                | 1.9%    | 1.7%                               | 1.9%    |
| Expected life of options (years)   | 6.25                                | 6.25    | 6.25                               | 6.25    |
| Expected annual dividend per share | \$ 0.00                             | \$ 0.00 | \$ 0.00                            | \$ 0.00 |

A summary of the Company's stock options for the nine months ended September 30, 2015 is as follows:

|  | Number of<br>Shares<br>(in thousands) | Weighted<br>Average Exercise<br>Price | Weighted<br>Average<br>Remaining<br>Contractual Life | Aggregate Intrinsic<br>Value<br>(in millions) |
|--|---------------------------------------|---------------------------------------|--|---|
| Balance at December 31, 2014                               | 10,020.7                              | \$ 5.02                               |  |   |
| Options granted  | 3,609.2                               | \$ 11.75                              |  |   |
| Options exercised  | (1,954.3)                             | \$ 5.49                               |  |   |
| Options forfeited  | (101.4)                               | \$ 5.71                               |  |   |
| Balance at September 30, 2015                              | 11,574.2                              | \$ 7.03                               | 7.5 years  | \$ 81.9                                       |
| Vested and unvested expected to vest<br>September 30, 2015 | 10,660.2                              | \$ 6.86                               | 7.4 years  | \$ 77.1                                       |
| Exercisable at September 30, 2015                          | 5,302.8                               | \$ 5.78                               | 5.9 years  | \$ 43.6                                       |

As of September 30, 2015, the total unrecognized compensation cost related to non-vested stock options granted was \$24.7 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 nine months ended September 30, 2015 is as follows:

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|  | Number of Shares<br>( in thousands) | Weighted<br>Average Grant<br>Date Fair<br>Value | Weighted<br>Average<br>Remaining Years | Aggregate Intrinsic<br>Value<br>( in millions) |
|--|-------------------------------------|---|--|--|
| Non-vested units as of December 31, 2014                   | 955                                 | \$ 2.28   |  |  |
| Granted  | 210                                 | \$ 12.94  |  |  |
| Vested   | (417)                               | \$ 10.90  |  |  |
| Forfeited  |                                     | \$  |  |  |
| Non-vested units as of September 30, 2015                  | 748                                 | \$ 5.26   | 1.0                                    | \$ 6.3   |
| Non-vested units expected to vest at<br>September 30, 2015 | 748                                 | \$ 5.26   | 1.0                                    | \$ 6.3   |

For the nine months ended September 30, 2015, 0.4 million of the RSUs vested and all non-vested units are expected to vest over their normal term. The total fair value of RSUs that vested and were released in the nine months ended September 30, 2015 was \$4.6 million.

As of September 30, 2015, there was \$2.7 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 1.0 year.

Table of Contents*Compensation Expense Related to Equity Awards*

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

|  | Three Months<br>Ended September 30, |          | Nine Months<br>Ended September 30, |          |
|--|-------------------------------------|----------|------------------------------------|----------|
|  | 2015                                | 2014     | 2015                               | 2014     |
| Equity compensation expense recognized in: |                                     |          |                                    |          |
| Research and development expense           | \$ 1,232                            | \$ 698   | \$ 3,224                           | \$ 1,950 |
| General and administrative expense         | 1,505                               | 953      | 3,705                              | 2,448    |
| Total equity compensation expense          | \$ 2,737                            | \$ 1,651 | \$ 6,929                           | \$ 4,398 |

**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 September 30, 2015. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three and nine months ended September 30, 2015.

*Success Fee Payable*

In connection with the Term Loan, as disclosed in Note 9. Short Term Borrowings and Long Term Debt, the Company recorded a contingent liability of \$0.4 million related to a success fee payable within six months of trigger event, with the trigger event being regulatory acceptance of NDA or MAA submission. The success fee payable to the lender was probability adjusted and discounted utilizing an appropriate discount rate and hence classified as Level 3. In June 2015, EMA validated the submission of the Company's MAA and the success fee became payable. The Company paid the success fee in connection with the re-payment of the debt in June 2015.

*Contingent Consideration Payable*

The contingent consideration payable resulted from acquisition of 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.

Significant assumptions used in the Scioderm preliminary valuation include (i) SD-101 clinical forecasts (ii) the probability and timing related to the achievement of certain developmental milestones and (iii) the discount rate which is a measure of the credit risk associated with settling the liability. The discount rate used was a range of rates between 0.4 and 1.2% as interpolated from the U.S. Treasury constant maturity yield curve over the time frame of anticipated milestone payments. The probability of achievement of clinical milestones is at 70% with milestone payments ranging from \$0 to \$269.9 million. The valuation will be performed quarterly. Gains and losses are included in the statement of operations. There is no assurance that any of the conditions for the milestone payments will be met.

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Significant assumptions used in the Callidus valuation include (i) ATB200 clinical forecasts (ii) the probability and timing related to the achievement of certain developmental milestones and (iii) the discount rate of 11.5% which is a measure of the credit risk associated with settling the liability. The probability of achievement of clinical milestones ranged from 24% to 95% with milestone payment outcomes ranging from \$0 to \$81 million. The valuation is performed quarterly. Gains and losses are included in the statement of operations. There is no assurance that any of the conditions for the milestone payments will be met.

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

The following table shows the change in the balance of contingent consideration payable for the three and nine months ended September 30, 2015 and 2014 respectively:

|   | Three Months<br>Ended September 30, |           | Nine Months<br>Ended September 30, |           |
|---|-------------------------------------|-----------|------------------------------------|-----------|
|   | 2015                                | 2014      | 2015                               | 2014      |
| Balance, beginning of the period  | \$ 11,800                           | \$ 10,800 | \$ 10,700                          | \$ 10,600 |
| Additions, from business acquisitions   | 269,884                             |           | 269,884                            |           |
| Unrealized change in fair value change during the period, included in Statement of Operations | 1,300                               | (600)     | 2,400                              | (400)     |
| Balance, end of the period  | \$ 282,984                          | \$ 10,200 | \$ 282,984                         | \$ 10,200 |

### *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 September 30, 2015 were \$0.5 million, are classified as trading securities and recorded at fair value with changes in the investments' fair value recognized in the period they occur. The asset investments consist of market exchanged mutual funds. During the three and nine months ended September 30, 2015, the unrealized loss was \$54 thousand and \$64 thousand respectively. 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 September 30, 2015, are identified in the following table (in thousands):

|                          | Level 1   | Level 2 | Total     |
|--------------------------|-----------|---------|-----------|
| <b>Assets:</b>           |           |         |           |
| Cash/ money market funds | \$ 19,439 | \$      | \$ 19,439 |

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|                                   |    |        |         |         |            |
|-----------------------------------|----|--------|---------|---------|------------|
| Corporate debt securities         |    |        | 127,650 |         | 127,650    |
| Commercial paper                  |    |        | 104,498 |         | 104,498    |
| Certificate of deposit            |    |        | 350     |         | 350        |
| Deferred compensation plan assets |    |        | 532     |         | 532        |
|                                   | \$ | 19,439 | \$      | 233,030 | \$ 252,469 |

|                                      | Level 1 | Level 2 | Level 3 | Total      |            |
|--------------------------------------|---------|---------|---------|------------|------------|
| <b>Liabilities:</b>                  |         |         |         |            |            |
| Contingent consideration payable     |         |         | 282,984 | 282,984    |            |
| Deferred compensation plan liability |         | 541     |         | 541        |            |
|                                      | \$      | \$      | 541     | \$ 282,984 | \$ 283,525 |

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A summary of the fair value of the Company's assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2014, are identified in the following table (in thousands):

|                           | Level 1   | Level 2    | Total      |
|---------------------------|-----------|------------|------------|
| <b>Assets:</b>            |           |            |            |
| Cash/ money market funds  | \$ 24,074 | \$         | \$ 24,074  |
| Corporate debt securities |           | 133,216    | 133,216    |
| Commercial paper          |           | 11,499     | 11,499     |
| Certificate of deposit    |           | 350        | 350        |
|                           | \$ 24,074 | \$ 145,065 | \$ 169,139 |

|                                      | Level 1 | Level 2 | Level 3   | Total     |
|--------------------------------------|---------|---------|-----------|-----------|
| <b>Liabilities:</b>                  |         |         |           |           |
| Contingent success fee payable       |         |         | 341       | 341       |
| Contingent consideration payable     |         |         | 10,700    | 10,700    |
| Deferred compensation plan liability |         | 124     |           | 124       |
|                                      | \$      | \$ 124  | \$ 11,041 | \$ 11,165 |

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

In December 2013, the Company entered into a credit and security agreement with a lending syndicate consisting of MidCap Funding III, LLC, Oxford Finance LLC, and Silicon Valley Bank. The Company drew \$15 million of the aggregate principal amount which bore interest at a rate per annum fixed at 8.5%. The Company made interest-only payments on the Term Loan from January 1, 2014. In June 2015, the Company paid off the outstanding balance of the term loan and in connection with this repayment the Company also paid a \$0.4 million exit fee and a \$0.4 million success fee due to the successful acceptance of the MAA in June 2015. The net loss on extinguishment of the debt was \$1.0 million and is included in the statement of operations for the nine months ended September 30, 2015.

**Note 10. Collaborative Agreements**

*GSK*

In November 2013, Amicus entered into the Revised Agreement with GlaxoSmithKline ( *GSK* ), pursuant to which Amicus has obtained global rights to develop and commercialize Galafold as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the Expanded Agreement entered into between Amicus and *GSK* in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from Amicus to *GSK*. For Galafold monotherapy, *GSK* is eligible to receive post-approval and sales-based milestones up to \$40 million, as well as tiered royalties in the mid-teens in eight major markets outside the United States.

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

*Biogen*

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

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



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

For the three and nine months ended September 30, 2014, the Company recognized \$0.3 and \$1.2 million, respectively, in Research Revenue for work performed under the cost sharing arrangement of the Biogen Agreement.

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

**Note 11. Restructuring Charges**

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

The following table summarizes the restructuring charges and utilization for the nine months ended September 30, 2015 (in thousands):

|                          | Balance as of<br>December 31, 2014 | Charges | Cash Payments | Adjustments | Balance as of<br>September 30,<br>2015 |
|--------------------------|------------------------------------|---------|---------------|-------------|--|
| Facilities consolidation | \$ 283                             | \$      | \$ (171)      | \$ 44       | \$ 156                                 |

**Note 12. 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:

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| (In thousands, except per share amounts)                     | Three Months Ended<br>September 30, |               | Nine Months Ended<br>September 30, |               |
|--|-------------------------------------|---------------|------------------------------------|---------------|
|  | 2015                                | 2014          | 2015                               | 2014          |
| <b>Historical</b>  |                                     |               |                                    |               |
| Numerator:   |                                     |               |                                    |               |
| Net loss attributable to common stockholders                 | \$ (37,800)                         | \$ (17,149)   | \$ (89,222)                        | \$ (47,706)   |
| Denominator:   |                                     |               |                                    |               |
| Weighted average common shares outstanding basic and diluted | \$ 118,724,882                      | \$ 78,889,346 | \$ 104,885,956                     | \$ 70,216,251 |

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 September 30, |        |
|---|---------------------|--------|
|   | 2015                | 2014   |
| Options to purchase common stock                  | 11,574              | 10,329 |
| Outstanding warrants, convertible to common stock |                     | 1,600  |
| Unvested restricted stock units                   | 748                 | 955    |
| Total number of potentially issuable shares       | 12,322              | 12,884 |

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**Note 13. 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.

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 14. Subsequent Events**

On October 1, 2015, the Company entered into a Note and Warrant Purchase Agreement with Redmile Capital Fund, LP and certain of its affiliates (Redmile) set forth in the Purchase Agreement, whereby it sold, on a private placement basis, (a) \$50.0 million aggregate principal amount of its unsecured promissory notes and (b) 1,349,998 warrants that have a term of five-years. 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 is 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 Sheet. The Company is in the process of evaluating the accounting treatment for the debt and the warrants.

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

**Overview**

We are a biopharmaceutical company focused on the discovery, development and commercialization of 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 (Zorblisa), a product candidate in late-stage development, is a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa (EB). We are also leveraging our biologics and Chaperone-Advanced Replacement Therapy (CHART) platform technologies to develop next-generation ERT products for Fabry, Pompe 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.

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

Our Fabry franchise strategy is to develop the pharmacological chaperone migalastat for all patients with Fabry disease - as a monotherapy for patients with amenable mutations (Galafold) and in combination with ERT for all other patients. We are also developing the proprietary and novel topical therapy Zorblisa for the treatment of skin blistering and lesions associated with all major subtypes of EB.

*Galafold for Fabry Disease*

We have completed two Phase 3 global registration studies (Study 011 and Study 012) of Galafold. We have reported Phase 3 data in both treatment-naïve patients (Study 011 or FACETS) and ERT switch patients (Study 012 or ATTRACT). Positive results from these studies have shown that treatment with Galafold has resulted in reductions in disease substrate, stability of kidney function, reductions in cardiac mass, and improvement in gastrointestinal symptoms in patients with amenable mutations.

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

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

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

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

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

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

- Subjects who switched from placebo to Galafold after month 6 demonstrated a statistically significant reduction in disease substrate, or kidney interstitial capillary GL-3, at month 12 ( $p=0.013$ ). Subjects who remained on migalastat demonstrated a durable reduction in kidney interstitial capillary GL-3, as well as a durable reduction in lyso-Gb3.
- Six months migalastat treatment was associated with a significant reduction in plasma lyso-Gb3 versus placebo ( $p=.0033$ ). The reduction remained stable following 6 additional months migalastat. A significant reduction in plasma lyso-Gb3 was found in patients switching from placebo to migalastat between 6 and 12-months ( $p<.0001$ ).
- Kidney function, as measured by estimated glomerular filtration rate ( eGFR ) and iohexol measured GFR ( mGFR ), remained stable following 18-24 months of treatment with Galafold in Study 011. Kidney function, as measured by eGFR, continued to remain stable in patients receiving migalastat in Study 011 for at least 18 months and continuing Galafold treatment in Study 041 for an average of 32 months. mGFR was not collected in Study 041.
- Reduction in cardiac mass, as measured by left ventricular mass index ( LVMI ), was statistically significant following treatment with migalastat for up to 36 months (average of 22 months) in patients in Study 011 and 041.
- There was a significant decrease in diarrhea (unadjusted  $p=0.03$ ) in patients treated with migalastat versus placebo during the 6-month double-blind phase (Stage 1). After 18-24 months of treatment with Galafold, significant improvements in diarrhea and indigestion were observed, in addition to favorable trends in reflux and constipation. Gastrointestinal symptoms were assessed using the Gastrointestinal Symptoms Rating Scale ( GSRS ), a validated instrument.
- Galafold was generally safe and well-tolerated.

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

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

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

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

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In June 2015, the European Medicines Agency ( EMA ) validated our Marketing Authorization Application ( MAA ) submission for Galafold and the Centralized Procedure has begun under Accelerated Assessment. The Committee for Medicinal Products for Human Use ( CHMP ) may shorten the MAA review period from 210 days, under standard review, to 150 days under Accelerated Assessment. The CHMP opinion is then reviewed by the European Commission, which generally issues a final decision on EU approval within three months. The MAA submission will be reviewed in the Centralized Procedure, which if authorized, provides a marketing license valid in all 28 EU member states. Once authorized, Amicus would then begin the country-by-country reimbursement approval process.

Following the MAA validation, the Company is also initiating the regulatory submission process in several additional geographies. In the United States, we plan to continue to work with the FDA to identify the optimal U.S. approval pathway for migalastat, and anticipate providing a regulatory update in the first half of 2016.

*Zorblisa for Epidermolysis Bullosa Disease*

Amicus is currently enrolling a Phase 3 study to evaluate Zorblisa in patients with all three major subtypes of *Epidermolysis Bullosa* – EB (Simplex, Recessive Dystrophic (RDEB) and Junctional [non-Herlitz]). Based on promising Phase 2 clinical data in EB patients, Zorblisa became the one of the first biotech drugs in 2013 to receive Breakthrough Therapy Designation by the (FDA) for the treatment of patients with EB. Zorblisa also has orphan drug designation from the FDA and EMA.

In individuals with EB, Zorblisa has shown acceleration in wound healing and closure of chronic wounds in a double-blind, randomized, placebo-controlled, dose-response Phase 2 study (SD-003) evaluating the efficacy and safety of different dosage strengths of Zorblisa.

*Migalastat Combination Programs for Fabry Disease*

We are developing migalastat in combination with ERT for fabry patients who do not have amenable mutations and cannot take monotherapy. We are developing a novel Fabry ERT cell-line and preclinical proof-of-concept studies co-formulating our Fabry ERT with migalastat will begin this quarter. As our internal novel ERT for Fabry has continued to advance, we plan to focus exclusively on this proprietary cell line for co-formulation with migalastat and no longer plan to conduct a co-administration study with commercially available ERT. We believe that further development of our own proprietary Fabry ERT represents the fastest and best path for bringing a novel therapy and meaningful improvements to Fabry patients with non-amenable mutations. We previously completed an open-label Phase 2 safety and pharmacokinetics study ( Study 013 ) that investigated two oral doses of migalastat (150 mg and 450 mg) co-administered with agalsidase beta or agalsidase alfa in males with Fabry disease. Unlike Study 011 and Study 012, patients in Study 013 were not required to have alpha-Gal A mutations amenable to chaperone therapy because, when co-administered with ERT, migalastat is designed to bind to and stabilize the exogenous enzyme in the circulation in any patient receiving ERT. Each patient received their current dose and regimen of ERT at one infusion. A single oral dose of migalastat (150 mg or 450 mg) was co-administered two hours prior to the next infusion of the same ERT at the same dose and regimen. Preliminary results from Study 013 showed increased levels of active alpha-Gal A enzyme levels in plasma and skin following co-administration compared to ERT alone.

*Next-Generation ERT for Pompe Disease*



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

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

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*Other Potential Alliances and Collaborations*

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

**Acquisition of 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.

As part of the merger agreement, we 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 Amicus shares. 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 Zorblisa is approved, EB qualifies as a rare pediatric disease and Amicus 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.

**Acquisition of Callidus Biopharma, Inc.**

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

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

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

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

There were no significant changes during the quarter ended September 30, 2015 to the items that we disclosed as our significant accounting policies and estimates described in Note 2 to the Company's financial statements as contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2014. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

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*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):

| Projects                            | Three Months ended<br>September 30, |      | Nine Months ended September<br>30, |      |
|-------------------------------------|-------------------------------------|------|------------------------------------|------|
|                                     | 2015                                | 2014 | 2015                               | 2014 |
| Third party direct project expenses |                                     |      |                                    |      |

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| Monotherapy Studies                                |    |        |    |        |    |        |    |        |
|--|----|--------|----|--------|----|--------|----|--------|
| Galafold (Fabry Disease Phase 3)                   | \$ | 3,798  | \$ | 3,141  | \$ | 11,736 | \$ | 8,961  |
| Combination (CHART) Studies                        |    |        |    |        |    |        |    |        |
| ATB200 + chaperone (Pompe Disease - Preclinical)   |    | 7,523  |    | 2,455  |    | 15,503 |    | 4,080  |
| Migalastat + chaperone (Fabry Disease Preclinical) |    | 461    |    | 284    |    | 1,587  |    | 902    |
| Neurodegenerative Diseases (Preclinical)           |    | 3      |    | 35     |    | 6      |    | 265    |
| Total third party direct project expenses          |    | 11,785 |    | 5,915  |    | 28,832 |    | 14,208 |
| Other project costs (1)                            |    |        |    |        |    |        |    |        |
| Personnel costs                                    |    | 6,598  |    | 4,361  |    | 18,133 |    | 12,811 |
| Other costs (2)                                    |    | 2,588  |    | 1,773  |    | 7,353  |    | 5,000  |
| Total other project costs                          |    | 9,186  |    | 6,134  |    | 25,486 |    | 17,811 |
| Total research and development costs               | \$ | 20,971 | \$ | 12,049 | \$ | 54,318 |    | 32,019 |

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

Table of Contents*Stock Option Grants*

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

We use the Black-Scholes option pricing model when estimating the value for stock-based awards. Use of a valuation model requires management to make certain assumptions with respect to selected model inputs. Expected volatility was based on our historical volatility since our initial public offering in May 2007. The expected life was determined using the simplified method as described in ASC Topic 718,

Accounting for Stock Compensation, which is the midpoint between the vesting date and the end of the contractual term. The risk-free interest rate was based on the U.S. Treasury yield in effect at the date of grant. Forfeitures are estimated based on expected turnover as well as a historical analysis of actual option forfeitures.

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

|                                    | Three Months<br>Ended September 30, |         | Nine Months<br>Ended September 30, |         |
|------------------------------------|-------------------------------------|---------|------------------------------------|---------|
|                                    | 2015                                | 2014    | 2015                               | 2014    |
| Expected stock price volatility    | 74.4%                               | 81.0%   | 75.4%                              | 81.3%   |
| Risk free interest rate            | 1.7%                                | 1.9%    | 1.7%                               | 1.9%    |
| Expected life of options (years)   | 6.25                                | 6.25    | 6.25                               | 6.25    |
| Expected annual dividend per share | \$ 0.00                             | \$ 0.00 | \$ 0.00                            | \$ 0.00 |

*Restricted Stock Units*

In 2014 and 2015, the Compensation Committee made awards of restricted stock units ( RSUs ) to certain employees of the Company. The RSUs were awarded under the Plan and are generally subject to graded vesting 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.

In April 2014, our Board of Director approved the Company's Restricted Stock Unit Deferral Plan (the Deferred Compensation Plan), which provides selected employees with an opportunity to defer receipt of RSUs until the first to occur of termination of the employee's employment or a date selected by the employee. Any RSUs deferred under the Deferred Compensation Plan would be fully vested once the original vesting conditions of the RSUs were satisfied. For the nine months ended September 30, 2015, 0.4 million RSUs have vested.

*Warrants*

The warrants issued in connection with our November 2013 securities and purchase agreement ( SPA ) were classified as equity. As part of the SPA, a total of 7.5 million common shares and 1.6 million warrants were issued at \$2.00 per share, for total cash received of \$15 million. The warrants were included in stockholder s equity and were initially measured at fair value of \$1.0 million using the Black Scholes valuation model. These warrants were fully exercised in June 2015 resulting in net proceeds of \$4.0 million during the second quarter of 2015.

On October 1, 2015, we entered into a Note and Warrant Purchase Agreement with Redmile Capital Fund, LP and certain of its affiliates ( Redmile ) set forth in the Purchase Agreement, 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. The promissory notes are recorded as due to related party on the Consolidated Balance Sheet. We are in the process of evaluating the accounting treatment for the debt and the warrants.

#### *Nonqualified Cash Deferral Plan*

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

The amounts deferred under the Deferral Plan are included in the non-current assets within the accompanying consolidated balance sheet. All of the investments held in the Deferral Plan are classified as trading securities and recorded at fair value with 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 September 30, 2015 Compared to Three Months Ended September 30, 2014*

*Revenue.* In September 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. This collaboration was ended in September 2014. For the three months ended September 30, 2015 and 2014, we recognized \$0 and \$0.3 million, respectively as Research Revenue for reimbursed research and development costs.

*Research and Development Expense.* Research and development expense was \$21.0 million during the three months ended September 30, 2015, representing an increase of \$9.0 million or 75% from \$12.0 million for the three months ended September 30, 2014. The increase in research and development costs was due primarily to increases in contract research and manufacturing, as well as increase in personnel costs of \$2.2 million. Contract manufacturing increased by \$5.3 million and contract research by \$0.6 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the ATB200 + chaperone program, migalastat + chaperone program and the Galafold program.

*General and Administrative Expense.* General and administrative expense was \$15.4 million for the three months ended September 30, 2015, representing an increase of \$10.1 million or 190.6% from \$5.3 million for the three months ended September 30, 2014. The increase was due to consulting fees of \$4.3 million, personnel costs of \$2.6 million, legal fees related to acquisition of \$1.0 million and recruitment fees of \$0.8 million. Included within this increase was \$4.9 million related to pre-commercial organization costs.

*Changes in Fair Value of Contingent Consideration Payable.* For the three months ended September 30, 2015, we recorded expense of \$1.3 million representing an increase of \$1.9 million or 316.7% from the \$0.6 million of gain for the three months ended September 30, 2014. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods and rates.

*Restructuring Charges.* Increase to the restructuring expense was \$7 thousand for three months ended September 30, 2015 as compared to \$15 thousand for the three months ended September 30, 2014, and was due to the change in fair value of future minimum lease payments.



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

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

*Interest Expense.* Interest expense was approximately \$17 thousand for the three months ended September 30, 2015, as compared to \$377 thousand for the three months ended September 30, 2014. The interest expense for the three months September 30, 2014 was incurred on the \$15 million loan secured in December 2013 that was paid in June 2015.

*Other Income/Expense.* Other income/expenses for the three months ended September 30, 2015 and September 30, 2014 was \$54 thousand and \$11 thousand, respectively. The change primarily included fair value changes to deferred compensation assets and fair value changes of the success fee payable related to the \$15 million loan. The \$15 million term loan was paid in full during the second quarter of 2015.

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*Nine Months Ended September 30, 2015 Compared to Nine Months Ended September 30, 2014*

*Revenue.* In September 2013, we entered into a collaboration agreement with Biogen to discover, develop and commercialize novel small molecules for the treatment of Parkinson's disease. This collaboration was ended in September 2014. For the nine months ended September 30, 2015 and 2014, we recognized \$0 and \$1.2 million, respectively, as Research Revenue for reimbursed research and development costs.

*Research and Development Expense.* Research and development expense was \$54.3 million during the nine months ended September 30, 2015, representing an increase of \$22.3 million or 69.7% from \$32.0 million for the nine months ended September 30, 2014. The increase in research and development costs was due primarily to increases in contract research and manufacturing, as well as increase in personnel costs of \$5.3 million. Contract manufacturing increased by \$10.2 million and contract research increased by \$4.9 million arising from the timing of studies and changes in research plans. These research plans included increased spending in the ATB200 + chaperone program, migalstatat + chaperone program and the Galafold program.

*General and Administrative Expense.* General and administrative expense was \$30.1 million for the nine months ended September 30, 2015, representing an increase of \$14.9 million or 98.0% from \$15.2 million for the nine months ended September 30, 2014. The increase was due to consulting fees of \$5.9 million, personnel costs of \$4.2 million, recruitment of \$2.1 million and legal fees related to acquisition of \$1.0 million. Included within this increase was \$8.1 million related to pre-commercial organization costs.

*Changes in Fair Value of Contingent Consideration Payable.* For nine months ended September 30, 2015, we recorded expense of \$2.4 million representing an increase of \$2.8 million or 700% from \$0.4 million of gain for the nine months ended September 30, 2014. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates.

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

*Restructuring Charges.* Increase to the restructuring expense was \$44 thousand for nine months ended September 30, 2015 as compared to a reduction in expense of \$74 thousand for the nine months ended September 30, 2014 and was due to the change in fair value of future minimum lease payments.

*Depreciation.* Depreciation expense was \$1.3 million for the nine months ended September 30, 2015, representing an increase of \$0.1 million or 8.3% as compared to \$1.2 million for the nine months ended September 30, 2014. The change was due to an increase in the amount of property, plant and equipment.

*Interest Income.* Interest income was \$0.6 million for the nine months ended September 30, 2015, representing an increase of \$0.5 million or 500% from \$0.1 million for the nine months ended September 30, 2014. The increase in interest income was due to the overall higher average cash and investment balances as a result of our financing transactions.

*Interest Expense.* Interest expense was approximately \$0.7 million for the nine months ended September 30, 2015, representing a decrease of \$0.4 million or 36.4% from \$1.1 million for the nine months ended September 30, 2014. The interest expense was lower due to the early retirement of the \$15 million secured loan.

*Other Expenses.* Other expenses for the nine months ended September 30, 2015 included charges of \$93 thousand as compared to \$30 thousand for the nine months ended September 30, 2014. The change was primarily from fair value changes of the success fee payable, related to the \$15 million secured loan and from fair value changes to deferred compensation assets.

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**Liquidity and Capital Resources**

*Source of Liquidity*

In June 2015, the Company issued a total of 19.5 million shares through a public offering at a price of \$13.25 per share. The offering generated gross proceeds of \$258.8 million. After deducting underwriting fees of \$15.5 million and other offering expenses of \$0.2 million, which included legal fees, the net proceeds of the offering were approximately \$243.0 million. The Company expects to use the net proceeds of the offering for investment in the global commercialization infrastructure for Galafold (migalastat) for Fabry disease, the continued clinical development of its product candidates and for other general corporate purposes.

In November 2014, we sold a total of 15.9 million shares of our common stock at a public offering price of \$6.50 per share. The offering generated gross proceeds of \$103.5 million. After deducting the underwriting fee of \$6.2 million and other offering expenses of \$0.1 million, which included legal fees, the net proceeds of the offering were approximately \$97.2 million.

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

As a result of our significant research and development expenditures and the lack of any approved products to generate product sales revenue, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have funded our operations principally with \$148.7 million of proceeds from redeemable convertible preferred stock offerings, \$576.4 million of gross proceeds from our stock offerings, \$130.0 million from investments by collaborators and non-refundable license fees from those collaborations.

In December 2013, we entered into a credit and security agreement with a lending syndicate which provided an aggregate of \$25 million credit available. We drew \$15 million of the aggregate principal amount in December 2013 and paid the outstanding balance of the loan in the second quarter of 2015.

During September 2015, we were in the process of negotiating a finance arrangement with Redmile Capital Fund, LP and certain of its affiliates (collectively, Redmile ). We received the proceeds related to the arrangement of \$50.0 million cash on September 28, 2015.

As of September 30, 2015, we had cash and cash equivalents and marketable securities of \$251.9 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 Operating Activities*

Net cash used in operations for the nine months ended September 30, 2015 was \$64.6 million, due primarily to the net loss for the nine months ended September 30, 2015 of \$89.2 million and non-cash items such as stock based compensation of \$6.9 million, the change in fair value of the contingent consideration of \$2.4 and the loss on the extinguishment of debt of \$1.0 million. In addition there was change in operating assets and liabilities of \$12.9 million. The change in operating assets and liabilities was due to an increase in accrued expenses of \$14.1 million, in prepaid assets of \$0.7 million and in other non-current assets of \$0.5 million.

Net cash used in operations for the nine months ended September 30, 2014 was \$37.2 million, due primarily to the net loss for the nine months ended September 30, 2014 of \$47.7 million and the change in operating assets and liabilities of \$5.3 million. The change in operating assets and liabilities consisted of a decrease in receivables from collaboration agreements of \$0.8 million; a decrease of \$3.4 million in prepaid assets primarily related to Net Operating Loss ( NOL ) receivable; an increase in accounts payable and accrued expenses of \$1.2 million, mainly related to program expenses as well as reclass of success fee from noncurrent liability to a current liability.

*Net Cash Used in Investing Activities*

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

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Net cash used in investing activities for the nine months ended September, 2014 was \$27.3 million. Net cash used in investing activities reflects \$75.1 million for the purchase of marketable securities and \$0.2 million for the acquisition of property and equipment, partially offset by \$48.0 million from the sale and redemption of marketable securities.

*Net Cash Provided by Financing Activities*

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

Net cash provided by financing activities for the nine months ended September 30, 2014 was \$40.5 million. Net cash provided reflects \$38.7 million in net proceeds from sales of common stock under our ATM agreement with Cowen, \$2.1 million from stock option exercises, partially offset by \$0.3 million for the payments of our secured loan agreement.

*Funding Requirements*

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

- the progress and results of our clinical trials of our drug candidates, including Galafold;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development as well as the cost of Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT for the treatment of lysosomal storage disorders;
- the future results of on-going or later clinical trials for Zorblisa, including our ability to obtain regulatory approvals and commercialize Zorblisa and market acceptance of Zorblisa;
- 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 at least 2016, if at all. In the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We may seek additional funding through public or private financings of debt or equity. We believe that our existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into 2017.

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

##### *Milestone Payments / Royalties*

Under our license agreements, if we owe royalties on net sales for one of our products to more than one licensor, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement.

Under the Revised Agreement, GSK is eligible to receive post-approval and sales-based milestones, as well as tiered royalties in the mid-teens in eight major markets outside the United States for Galafold. In addition, because we reacquired worldwide rights to Galafold, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement. We will owe royalties to Mt. Sinai School of Medicine in addition to those owed to GSK.

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 Zorblisa is approved, EB qualifies as a rare pediatric disease and Amicus 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 its Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Select Market for the ten

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(10) trading days immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that the Company is permitted to, but chooses not to, satisfy in Common Stock), as a result of the terms of the merger agreement, the rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash.

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



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***Recent Accounting Pronouncements***

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

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

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At September 30, 2015, we held \$251.9 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 the fair value of our investments.

We have operated primarily in the United States, although we do conduct some clinical activities outside the United States. While most expenses are paid in U.S. dollars, there are minimal payments made in local foreign currency. If exchange rates undergo a change of 10%, we do not believe that it would have a material impact on our results of operations or cash flows.

**ITEM 4. CONTROLS AND PROCEDURES**

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

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



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**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

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 consolidated action.

We believe that we have meritorious defenses and intend to defend the lawsuit vigorously. 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, we could be forced to expend significant resources in the defense of this lawsuit 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, 2014 and on Form 10-Q for the period ended June 30, 2015.

Table of Contents**ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS****Recent Sales of Unregistered Securities**

None.

**Issuer Purchases of Equity Securities**

The following table sets forth purchases of our common stock for the three months ended September 30, 2015:

| Period                                 | (a) Total number of shares purchased | (b) Average Price Paid per Share | (c) Total number of shares purchased as part of publicly announced plans or programs | (d) Maximum number of shares that may yet be purchased under the plans or programs |
|--|--------------------------------------|----------------------------------|--|--|
| July 1, 2015 - July 31, 2015           | 4,544                                | \$ 14.10                         |  | 7,956  |
| August 1, 2015 - August 31, 2015       |                                      |                                  |  |  |
| September 1, 2015 - September 30, 2015 |                                      |                                  |  |  |
| Total                                  | 4,544                                |                                  |  | 7,956  |

Pursuant to a restricted stock award dated April 10, 2014 between Amicus Therapeutics and certain employee recipients, certain employees were granted restricted stock units ( RSU ). The RSU s vested in July 2015 and the remainder will vest in December 2015, subject generally to the employee s continued employment with the Company. In order to comply with the minimum statutory federal tax withholding rate of 25%, 1.45% for Medicare plus 6.2% for Social Security where applicable, and state tax withholding of 9.9%, the employees surrendered to us a portion of their vested shares on the vesting date, representing between 36.36-42.56% of the total value of the shares then vested.

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**ITEM 4. MINE SAFETY DISCLOSURES**

None.

**ITEM 5. OTHER INFORMATION**

None.

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**ITEM 6. EXHIBITS**

| <b>Exhibit<br/>Number</b> | <b>Description</b>   |
|---------------------------|--|
| 1.1(1)                    | Underwriting Agreement dated June 11, 2015, by and amount Amicus Therapeutics, Inc., J.P. Morgan Securities LLC and Goldman, Sachs & Co., as representatives of the several underwriters set forth on Schedule I thereto.  |
| 2.1(2)                    | Agreement and Plan of Merger by and among Amicus Therapeutics, Inc., Titan Merger Sub Corp., Scioderm, Inc. and Fortis Advisors LLC, as Shareholders Agent.  |
| 2.2(3)                    | Amendment to Agreement and Plan of Merger, dated September 30, 2015, by and among Amicus Therapeutics, Inc., Titan Merger Sub Corp., Scioderm, Inc., Fortis Advisors LLC, as Shareholders Agent and certain Shareholders of Scioderm, Inc.   |
| 3.1(4)                    | Restated Certificate of Incorporation  |
| 3.2(5)                    | Certificate of Amendment to the Company s Restated Certificate of Incorporation, as amended.   |
| 3.3 (6)                   | Amended and Restated By-laws   |
| 10.1 (7)                  | First Amendment to Credit and Security Agreement, dated April 27, 2015 by and among Amicus Therapeutics, Inc. and the other entities shown as signatories thereto as a Borrower, the financial institutions or other entities from time to time parties as lenders, and Midcap Funding III Trust, as agent.  |
| 10.2 (8)                  | First Amendment to Lease, dated September 9, 2015, by and between Cedar Brook 3 Corporate Center, L.P. and Amicus Therapeutics, Inc.   |
| 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 September 30, 2015, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements. |

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- (1) Incorporated by reference to Exhibit 1.1 to our Current Report on Form 8-K filed on June 12, 2015.
  - (2) Incorporated by reference to Exhibit 2,1 to our Current Report on Form 8-K filed on September 3, 2015.
  - (3) Incorporated by reference to Exhibit 2.2 to our Current Report on Form 8-K filed on September 30, 2015.
  - (4) Incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on August 5, 2015.
  - (5) Incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on August 5, 2015.

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- (6) Incorporated by reference to Exhibit 3.3 to our Registration Statement on Form S-1.
- (7) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on April 28, 2015.
- (8) Incorporated by reference to Exhibit 10.1 to our Current Report on Form 8-K filed on September 9, 2015.

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**SIGNATURES**

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

AMICUS THERAPEUTICS, INC.

Date: November 4, 2015

By:

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

Date: November 4, 2015

By:

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



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