AMICUS THERAPEUTICS INC

FORM 10-Q
(Quarterly Report)

Filed 05/09/17 for the Period Ending 03/31/17

Address	1 CEDAR BROOK DRIVE CRANBURY, NJ 08512
Telephone	(609) 662-2000
CIK	0001178879
Symbol	FOLD
SIC Code	2834 - Pharmaceutical Preparations
Industry	Pharmaceuticals
Sector	Healthcare
Fiscal Year	12/31

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2017

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	**71-0869350**
(State or Other Jurisdiction of Incorporation or Organization)	(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, smaller reporting company, or an emerging growth company. See definition of "large accelerated filer," accelerated filer", "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☒	Accelerated filer ☐
Non-accelerated filer ☐ (Do not check if a smaller reporting company)	Smaller reporting company ☐
	Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐ or

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

The number of shares outstanding of the registrant's common stock, $.01 par value per share, as of April 25, 2017 was 142,916,720 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly P eriod Ended March 31, 2017

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

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

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

- the progress and results of our clinical trials of our drug candidates;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new Fabry enzyme replacement therapy (“ERT”) cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage disorders (“LSDs”);
- the future results of on-going or later clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and obtain market acceptance of SD-101;
- the future results of on-going preclinical research and later clinical trials for cyclin-dependent kinase-like 5 (“CDKL5”), including our ability to obtain regulatory approvals and commercialize CDKL5 and obtain market acceptance for CDKL5;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to obtain reimbursement for migalastat HCI;
- our ability to obtain market acceptance of migalastat HCl in the European Union (the “EU”);
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property-related claims;
- the extent to which we acquire or invest in businesses, products and technologies;
- our ability to successfully integrate our acquisitions of Scioderm, Inc. (“Scioderm”) and MiaMed, Inc. (“MiaMed”) and their products and technologies into our business, including the possibility that the expected benefits of the transactions 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, as amended, for the year ended December 31, 2016, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make.

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

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

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

	March 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 44,755	$ 187,026
Investments in marketable securities	235,087	143,325
Accounts receivable	1,875	1,304
Inventories	3,698	3,416
Prepaid expenses and other current assets	10,792	4,993
Total current assets	296,207	340,064
Property and equipment, less accumulated depreciation of $13,316 and $12,495 at March 31, 2017 and December 31, 2016, respectively	9,745	9,816
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	2,932	2,468
Total Assets	$ 993,381	$ 1,036,845
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 39,645	$ 41,008
Deferred reimbursements, current portion	13,850	13,850
Contingent consideration payable, current portion	56,101	56,101
Total current liabilities	109,596	110,959
Deferred reimbursements	21,906	21,906
Convertible notes	156,859	154,464
Contingent consideration payable	218,199	213,621
Deferred income taxes	173,820	173,771
Other non-current liability	2,223	1,973
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 250,000,000 shares authorized, 142,829,530 and 142,691,986 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	1,482	1,480
Additional paid-in capital	1,126,148	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax expense of $1,293 at March 31, 2017 and December 31, 2016	1,487	1,945
Unrealized gain on available-for securities	185	102
Warrants	16,076	16,076
Accumulated deficit	(834,600)	(779,608)
Total stockholders' equity	310,778	360,151
Total Liabilities and Stockholders' Equity	$ 993,381	$ 1,036,845

See accompanying notes to consolidated financial statements

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

	Three Months Ended March 31, 2017	Three Months Ended March 31, 2016
Revenue:		
Net product sales	$ 4,169	$ —
Cost of goods sold	775	—
Gross Profit	3,394	—
Operating Expenses:		
Research and development	30,876	23,425
Selling, general and administrative	19,132	15,701
Changes in fair value of contingent consideration payable	4,578	3,152
Restructuring charges	—	50
Depreciation	823	673
Total operating expenses	55,409	43,001
Loss from operations	(52,015)	(43,001)
Other income (expenses):		
Interest income	759	307
Interest expense	(4,290)	(945)
Other income (expense)	610	(52)
Loss before income tax benefit	(54,936)	(43,691)
Income tax expense	(56)	—
Net loss attributable to common stockholders	$ (54,992)	$ (43,691)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.39)	$ (0.35)
Weighted-average common shares outstanding — basic and diluted	142,770,629	125,178,517

See accompanying notes to consolidated financial statements

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

	Three Months Ended March 31, 2017	Three Months Ended March 31, 2016
Net loss	$ (54,992)	$ (43,691)
Other comprehensive gain/ (loss):		
Foreign currency translation adjustment	(458)	(65)
Unrealized gain on available-for-sale securities	83	229
Other comprehensive (loss)/ income	(375)	164
Comprehensive loss	$ (55,367)	$ (43,527)

See accompanying notes to consolidated financial statements

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

	Three Months Ended March 31,	
	2017	2016
Operating activities		
Net loss	$ (54,992)	$ (43,691)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	2,403	451
Depreciation	823	673
Stock-based compensation	6,030	4,283
Restructuring charges	—	50
Change in fair value of derivative liability	(163)	—
Non-cash changes in the fair value of contingent consideration payable	4,578	3,152
Foreign currency remeasurement loss	(604)	—
Non-cash deferred taxes	49	—
Changes in operating assets and liabilities:		
Accounts receivable	(512)	—
Inventories	(243)	—
Prepaid expenses and other current assets	(5,846)	(94)
Other non-current assets	(459)	(377)
Account payable and accrued expenses	(1,271)	(9,763)
Non-current liabilities	363	380
Net cash used in operating activities	(49,844)	(44,936)
Investing activities		
Sale and redemption of marketable securities	69,443	53,157
Purchases of marketable securities	(161,123)	(50,721)
Purchases of property and equipment	(732)	(2,878)
Net cash used in investing activities	(92,412)	(442)
Financing activities		
Payment of capital leases	(71)	—
Purchase of vested restricted stock units	(261)	(657)
Proceeds from exercise of stock options	265	155
Payment of deferred financing fees	(28)	—
Net cash used in financing activities	(95)	(502)
Effect of exchange rate changes on cash and cash equivalents	80	(95)
Net decrease in cash and cash equivalents	**(142,271)**	**(45,975)**
Cash and cash equivalents at beginning of year/ period	187,026	69,485
Cash and cash equivalents at end of year/period	**$** 44,755	**$ 23,510**
Supplemental disclosures of cash flow information		
Cash paid during the period for interest	$ 12	$ —

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.

Notes to the Consolidated Financial Statements
(Unaudited)

Note 1. Description of Business

Corporate Information, Status of Operations, and Management Plans

Amicus Therapeutics, Inc. (the "Company") is a global patient-focused biotechnology company engaged in the discovery, development, and commercialization of a diverse set of novel treatments for patients living with devastating rare and orphan diseases. The lead product, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement of therapy ("ERT") for Fabry disease.

In May 2016, the Company announced that the European Commission ("EC") had granted full approval for the oral small molecule pharmacological chaperone Galafold™ (migalastat) as a first-line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. The approved label includes 313 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. The Company commenced commercial shipments of Galafold in the EU in the second quarter of 2016 and recognized net product sales of $4.2 million in the three months ended March 31, 2017.

Also in the pipeline, SD-101 is a product candidate in late-stage development, as a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa ("EB"). The Company is also leveraging its biologics and Chaperone-Advanced Replacement Therapy ("CHART™") platform technologies to develop novel ERT products for Pompe disease, Fabry disease, and potentially other lysosomal storage disorders ("LSDs"). The Company is also investigating preclinical and discovery programs in other rare and devastating diseases including cyclin-dependent kinase-like 5 ("CDKL5") deficiency. The Company believes that the platform technologies and advanced product pipeline uniquely position the Company at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

The Company had an accumulated deficit of approximately $834.6 million at March 31, 2017 and anticipates incurring losses through the fiscal year ending December 31, 2017 and beyond. The Company has been able to fund its operating losses to date through stock offering, debt issuances, and payments from partners during the terms of the collaboration agreements and other financing arrangements.

The Company believes that its existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into the second half of 2018.

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 Regulation S-X. Accordingly, they do not include all of the information and disclosures required by U.S. GAAP 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, as amended, for the year ended December 31, 2016. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K, as amended for the year ended December 31, 2016.

Consolidation

The consolidated financial statements include the accounts of the Company and its subsidiaries. Intercompany accounts and transactions have been eliminated in consolidation.

Foreign Currency Transactions

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

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016, the Company entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company does not designate this forward contract as a hedging instrument under applicable accounting guidance and, therefore, changes in fair value are recorded as other expense in the Consolidated Statements of Operations.

Use of Estimates

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

Concentration of Credit Risk

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

The Company is subject to credit risk from its accounts receivable related to its product sales of Galafold. The majority of the Company's accounts receivable at March 31, 2017 have arisen from product sales primarily in Germany. The Company will periodically assess the financial strength of its customers to establish allowances for anticipated losses, if any. For accounts receivable that have arisen from named patient sales, the payment terms are predetermined and the Company evaluates the creditworthiness of each customer on a regular basis. To date, the Company has not incurred any credit losses.

Significant Accounting Policies

There have been no material changes to the Company's significant accounting policies during the three months ended March 31, 2017, 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, as amended, for the year ended December 31, 2016. 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 persuasive evidence of an arrangement exists, title to product and associated risk of loss has passed to the customer, which is typically upon receipt by the end customer, the price is fixed or determinable, collection of the amounts due are reasonably assured and the Company has no further performance obligations.

The Company's net product sales consist solely of sales of Galafold for the treatment of Fabry disease in the EU. The Company has recorded revenue on sales where Galafold is available either on a commercial basis or through a reimbursed early access program. Orders for Galafold are generally received from pharmacies and the ultimate payor is typically a government authority.

The Company records revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

Inventories and Cost of Goods Sold

Until regulatory approval of Galafold, the Company expensed all manufacturing costs related to Galafold as research and development expense. Upon regulatory approval, the Company began capitalizing costs related to the purchase and manufacture of Galafold.

Inventories are stated at the lower of cost and net realizable value, determined by the first-in, first-out method. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity as well as product shelf-life. In evaluating the recoverability of inventories produced, the probability that revenue will be obtained from the future sale of the related inventory is considered and inventory value is written down for inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statements of operations.

Cost of goods sold includes the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, provisions for excess and obsolete inventory, as well as royalties payable. A portion of the inventory available for sale was expensed as research and development costs prior to regulatory approval and as such the cost of goods sold and related gross margins are not necessarily indicative of future cost of goods sold and gross margin.

Fair Value Measurements

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

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

Contingent Liabilities

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

Recent Accounting Pronouncements

In March 2017, the FASB issued ASU 2017-08, *Receivables—Nonrefundable Fees and Other Costs (Subtopic 310-20), Premium Amortization on Purchased Callable Debt Securities.* The amendments shorten the amortization period for certain callable debt securities held at a premium. Specifically, the amendments require the premium to be amortized to the earliest call date. The amendments do not require an accounting change for securities held at a discount; the discount continues to be amortized to maturity. The ASU is effective for public business entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. For other entities, the amendments are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted, including adoption in an interim period. If an entity early adopts in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. The amendments should be applied on a modified retrospective basis, with a cumulative-effect adjustment directly to retained earnings as of the beginning of the period of adoption. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In January 2017, the FASB issued ASU 2017-04 , *Intangibles - Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment* . To simplify the subsequent measurement of goodwill, ASU 2017-04 eliminates Step 2 from the goodwill impairment test. The annual, or interim, goodwill impairment test is performed by comparing the fair value of a reporting unit with its carrying amount. An impairment charge should be recognized for the amount by which the carrying amount exceeds the reporting unit's fair value; however, the loss recognized should not exceed the total amount of goodwill allocated to that reporting unit. In addition, income tax effects from any tax deductible goodwill on the carrying amount of the reporting unit should be considered when measuring the goodwill impairment loss, if applicable. ASU 2017-04 also eliminates the requirements for any reporting unit with a zero or negative carrying amount to perform a qualitative assessment and, if it fails that qualitative test, to perform Step 2 of the goodwill impairment test. An entity still has the option to perform the qualitative assessment for a reporting unit to determine if the quantitative impairment test is necessary. ASU 2017-04 should be applied on a prospective basis. The nature of and reason for the change in accounting principle should be disclosed upon transition. A public business entity that is a U.S. SEC filer should adopt ASU 2017-04 for its annual or any interim goodwill impairment tests in fiscal years beginning after December 15, 2019. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In January 2017, the FASB issued ASU 2017-01, *Business Combinations (Topic 805): Clarifying the Definition of a Business.* This Accounting Standards Update clarifies the definition of a business. The amendments affect all companies and other reporting organizations that must determine whether they have acquired or sold a business. The amendments in this update are effective for public companies for annual periods beginning after December 15, 2017, including interim periods within those periods. Early adoption is permitted under certain circumstances. The amendments should be applied prospectively as of the beginning of the period of adoption. The Company has not completed review of the impact of this guidance and does not expect this new guidance to have a material impact on its consolidated financial statements.

In October 2016, the FASB issued ASU 2016-16, *Income Taxes (Topic 740): Intra-Entity Transfers of Assets Other Than Inventory* . This Accounting Standards Update requires an entity to recognize the income tax consequences of an intra-entity transfer of an asset other than inventory when the transfer occurs. The amendments eliminate the exception for an intra-entity transfer of an asset other than inventory. The amendments in this Update are effective for public business entities for annual periods beginning after December 15, 2017, including interim reporting periods within those annual reporting periods. Early adoption is permitted for all entities in the first interim period if an entity issues interim financial statements. The Company is currently assessing the impact that this standard will have on its consolidated financial statements. The Company has not completed review of the impact of this guidance and does not expect this new guidance to have a material impact on its consolidated financial statements.

In March 2016, the FASB issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting* . The amendments are intended to improve the accounting for employee share-based payments and affect all organizations that issue share-based payment awards to their employees. Several aspects of the accounting for share-based payment award transactions are simplified, including: (a) income tax consequences; (b) classification of awards as either equity or liabilities; and (c) classification on the statement of cash flows. For public companies, the amendments are effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. The Company adopted ASU 2016-09 on January 1, 2017. Due to the Company's history of operating losses, the adoption did not result in changes to the Company's Net loss or Retained earnings and the classification of excess tax benefits on the statement of cash flows for prior periods have not been adjusted. In connection with the adoption of ASU 2016-9, the Company made a policy election to continue its methodology for the development and application of its forfeiture rate.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* . This update requires the recognition of lease assets and lease liabilities on the balance sheet for all lease obligations and disclosing key information about leasing arrangements. This update requires the recognition of lease assets and lease liabilities by lessees for those leases classified as operating leases under previous generally accepted accounting principles. This update will be effective for the Company for all annual and interim periods beginning after December 15, 2018, including interim periods within those fiscal years. Early application is permitted for all public business entities and all nonpublic business entities upon issuance. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In July 2015, the FASB issued ASU 2015-11, Inventory (Topic 330): *Simplifying the Measurement of Inventory,* which requires an entity to measure in scope inventory at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The amendments apply to inventory that is measured using first-in, first-out (FIFO) or average cost. The ASU is effective for public business entities for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. A reporting entity should apply the amendments prospectively with earlier application permitted as of the beginning of an interim or annual reporting period. The Company adopted this guidance as of January 1, 2017 and the adoption did not have any impact on its consolidated financial statements.

In May 2014, FASB issued ASU 2014-09, *Revenue from Contracts with Customers* which along with amendments issued in 2015 and 2016, will replace substantially all current US GAAP guidance on this topic and eliminate industry-specific guidance. Early adoption of this standard is permitted but not before the original effective date for all annual periods and interim reporting periods beginning after December 15, 2017. The guidance permits two methods of adoption: full retrospective method (retrospective application to each prior reporting period presented) or modified retrospective method (retrospective application with the cumulative effect of initially applying the guidance recognized at the date of initial application and providing certain additional disclosures). To complete its assessment of the impact of the standard to its financial statements, the Company continues to assess all implications of this standard, method of adoption and related financial disclosures. Additionally, the Company continues to monitor modifications, clarifications and interpretations issued by the FASB that may impact its assessment.

3. Acquisitions

Asset acquisition of MiaMed, Inc.

In July 2016, the Company entered into an Agreement and Plan of Merger (the “MiaMed Agreement”) with MiaMed, Inc., (“MiaMed”). MiaMed is a pre-clinical biotechnology company focused on developing protein replacement therapy for CDKL5 and related diseases. Under the terms of the MiaMed Agreement, the former holders of MiaMed’s capital stock received an aggregate of $6.5 million, comprised of (i) approximately $1.8 million in cash (plus MiaMed’s cash and cash equivalents at closing and less any of MiaMed’s unpaid third-party fees and expenses related to the transaction), and (ii) 825,603 shares of the Company’s common stock, par value $0.01 per share (“Common Stock”). In addition, the Company also agreed to pay up to an additional $83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of $89.5 million. The Company evaluated the transaction based on the guidance of Accounting Standard Codification (“ASC”) 805 *, Business Combinations* and concluded that it only acquired inputs and did not acquire any processes. The Company will need to develop its own processes in order to produce an output. Therefore, the Company accounted for the transaction as an asset acquisition and accordingly $6.5 million was expensed to research and development.

Acquisition of Scioderm, Inc.

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

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

The fair value of the contingent consideration payments on the acquisition date was $259.0 million. This was an estimate based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions included a range of discount rates between 0.4% and 1.1% as interpolated from the U.S. Treasury constant maturity yield curve over the time frame for clinical and regulatory milestones and a range of discount rates between 1.0% and 2.2% for revenue-based milestones. The range of outcomes and assumptions used to develop these estimates have been updated to better reflect the probability of certain milestone outcomes and updated timelines related to clinical development and anticipated approval assumptions as of March 31, 2017, without limitation, the milestone payments projected for 2017 (See "— Note 9. Assets and Liabilities Measured at Fair Value", for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). In April 2016, while the total clinical and regulatory approval milestone payments remain unchanged at $361 million, the allocation between the clinical and regulatory approval milestone payments were revised as follows: clinical milestones of up to $81 million and regulatory approval milestones of up to $280 million. The commercial milestone payments of up to $257 million remained unchanged.

At the end of the first quarter of 2017, the Company achieved 100% enrollment in the Phase 3 clinical study of SD-101 and the milestone payment of $10 million due for this event, was paid in April 2017 and was included in Contingent Consideration payable, current portion on the Consolidated Balance Sheet as of March 31, 2017.

The Company determined the fair value of the contingent consideration to be $264.0 million at March 31, 2017, of which $56.1 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of approximately $4.0 million for the three months ended March 31, 2017. The expense is recorded in the Consolidated Statement of Operations as the change within fair value of contingent consideration payable.

For additional information, see "— Note 4. Goodwill and Intangible Assets."

The purchase price allocation was completed as of December 31, 2015 and a substantial portion of the assets acquired consisted of intangible assets related to SD-101. The Company determined that the estimated acquisition-date fair value of the indefinite lived IPR&D related to the SD-101 was $463.7 million.

4. Goodwill and IPR&D

In connection with the acquisitions, the Company has recognized goodwill of $197.8 million. The following table represents the changes in goodwill for the three months ended March 31, 2017:

	(in millions)
Balance at December 31, 2016	$ 197.8
Change in goodwill	—
Balance at March 31, 2017	$ 197.8

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

The following table represents the changes in IPR&D for the three months ended March 31, 2017:

		(in millions)
Balance at December 31, 2016	$	486.7
Change in IPR&D		—
Balance at March 31, 2017	$	486.7

Goodwill and intangible assets are assessed annually for impairment on October 1 and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value.

Note 5. Cash, Money Market Funds and Marketable Securities

As of March 31, 2017, the Company held $44.8 million in cash and cash equivalents and $235.1 million of available-for-sale securities which are reported at fair value on the Company's Consolidated Balance Sheets. 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.

The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating. Investments that have original maturities or greater than 3 months but less than 1 year are classified as short-term and investments with maturities that are greater than 1 year are classified as long-term.

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016 the Company entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company does not designate these forward contracts as hedging instruments under applicable accounting guidance and, therefore, changes in fair value are recorded within other income (expense) in the Consolidated Statements of Operations, with the corresponding liability in current liabilities on the Consolidated Balance Sheet. For the three months ended March 31, 2017, the Company recognized a gain of $0.2 million, related to the derivative instruments not designated as hedging instruments in other expense in the Consolidated Statements of Operations and the corresponding liability of $0.1 million is recorded as other current liability in the Consolidated Balance Sheets.

Cash and available-for-sale securities are all current unless mentioned otherwise and consisted of the following as of March 31, 2017 and December 31, 2016 (in thousands):

	As of March 31, 2017			
	Cost	Gross unrealized Gain	Gross unrealized Loss	Fair Value
Cash balances	$ 44,755	$ —	$ —	$ 44,755
Corporate debt securities, current portion	124,061	5	(38)	124,028
Commercial paper	110,441	218	—	110,659
Money market	350	—	—	350
Certificate of deposit	50	—	—	50
	$ 279,657	$ 223	$ (38)	$ 279,842
Included in cash and cash equivalents	$ 44,755	$ —	$ —	$ 44,755
Included in marketable securities	234,902	223	(38)	235,087
Total cash and marketable securities	$ 279,657	223	$ (38)	$ 279,842

	As of December 31, 2016			
	Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash balances	$ 187,026	$ —	$ —	$ 187,026
Corporate debt securities, current portion	74,564	2	(31)	74,535
Commercial paper	68,258	132	—	68,390
Money market	350	—	—	350
Certificate of deposit	50	—	—	50
	$ 330,248	$ 134	$ (31)	$ 330,351
Included in cash and cash equivalents	$ 187,026	$ —	$ —	$ 187,026
Included in marketable securities	143,222	134	(31)	143,325
Total cash and marketable securities	$ 330,248	134	$ (31)	$ 330,351

For the three months ended March 31, 2017 and the year ended December 31, 2016, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available for sale securities as of March 31, 2017 and December 31, 2016 reflect temporary impairments that have been in a loss position for less than twelve months and as such are recognized in other comprehensive gain/ (loss). The fair value of these available for sale securities in unrealized loss positions was $101.3 million and $58.7 million as of March 31, 2017 and December 31, 2016, respectively.

The Company holds available-for-sale investment securities which are reported at fair value on the Company's balance sheet. Unrealized holding gains and losses are reported within accumulated other comprehensive income ("AOCI") in the statements of comprehensive loss.

Note 6. Inventories

Inventories consist of work in process and finished goods related to the manufacture of Galafold. The following table summarizes the components of inventories at March 31, 2017 (in thousands):

	March 31, 2017	December 31, 2016
Work-in-process	$ 3,473	$ 3,308
Finished goods	225	108
Total inventories	$ 3,698	$ 3,416

Inventory manufactured prior to commercialization was expensed to research and development. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity, as well as product shelf-life. In evaluating the recoverability of inventories produced, the Company considers the probability that revenue will be obtained from the future sale of the related inventory. Inventory becomes obsolete when it has aged past its shelf-life, cannot be recertified and is no longer usable or able to be sold, or the inventory has been damaged. In such instances, a full reserve is taken against such inventory. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statement of operations. There have been no write-downs of inventory from the time inventory was first capitalized.

Note 7. Debt Instruments

2016 Convertible Debt

On December 21, 2016, the Company issued at par value $250 million aggregate principal amount of unsecured Convertible Senior Notes due 2023 (the "Convertible Notes"), which included the exercise in full of the $25 million over-allotment option granted to the initial purchasers of the Notes, in a private offering to qualified institutional buyers pursuant to Rule 144A under the Securities Act. Interest is payable semiannually on June 15 and December 15 of each year, beginning on June 15, 2017. The Notes will mature on December 15, 2023, unless earlier repurchased, redeemed, or converted in accordance with their terms. The Notes are convertible at the option of the holders, under certain circumstances and during certain periods, into cash, shares of the Company's Common Stock or a combination thereof and may be settled as described below. The net proceeds from the Note Offering were $243.1 million, after deducting fees and estimated expenses payable by the Company. In addition, the Company used approximately $13.5 million of the net proceeds from the issuance of the Convertible Notes to pay the cost of the capped call transactions ("Capped Call Confirmations") that the Company entered into in connection with the issuance of the Convertible Notes.

The Convertible Notes are governed by an indenture dated December 21, 2016 (the "Indenture") by and between the Company and Wilmington Trust, National Association, as trustee.

The Convertible Notes are initially convertible into approximately 40,849,675 shares of the Company's Common Stock under certain circumstances prior to maturity at a conversion rate of 163.3987 shares per $1,000 principal amount of Convertible Notes, which represents a conversion price of approximately $6.12 per share of Common Stock, subject to adjustment under certain conditions. Holders may convert their Convertible Notes at their option at specified times prior to the maturity date of December 15, 2023, only if:

- during any fiscal quarter commencing after March 31, 2017, if the last reported sale price of the Company's Common Stock for at least 20 trading days in the period of 30 consecutive trading days ending on the last trading day of the immediately preceding fiscal quarter is equal to or more than 130% of the conversion price of the Convertible Notes on the last day of such preceding fiscal quarter;
- a Holder submits its Convertible Notes for conversion during the five business day period following any five consecutive trading day period in which the trading price for the Convertible Notes, per $1,000 principal amount of the Convertible Notes, for each such trading day was less than 98% of the product of the last reported sale price of the Company's Common Stock and the conversion rate of the Convertible Notes on such date;
- the Company issues to all or substantially all of the holders of Common Stock rights options or warrants entitling them for a period of not more than 60 calendar days after the date of such issuance to subscribe for or purchase shares of the Common Stock, at a price per share less than the average of the Last Reported Sale Prices of the Common Stock for the 10 consecutive Trading Day period ending on, and including, the Trading Day immediately preceding the date of announcement of such issuance or distributes to all or substantially all holders of the Common Stock the Company's assets, debt securities or rights to purchase the Company's securities which distribution has a per share value of exceeding 10% of the Last Reported Sale Price of the Common Stock on the Trading Day immediately preceding the date of announcement of such distribution
- the Company enters into specified corporate transactions; or
- the Company has had a call for redemption, the holder can convert up until the second trading day immediately preceding the redemption date

The Convertible Notes will be convertible, at the option of the note holders, regardless of whether any of the foregoing conditions have been satisfied, on or after September 15, 2023 at any time prior to the close of business on the second scheduled trading day immediately preceding the stated maturity date of December 15, 2023.

Upon the occurrence of a make-whole fundamental change or if the Company call all or any portion of the Convertible Notes for redemption prior to July 1, 2020, the Company will, in certain circumstances, increase the conversion rate by a number of additional shares for a holder that elects to convert its Convertible Notes in connection with such make-whole fundamental change or during the related redemption period.

Upon conversion, the Company may pay cash, shares of the Company's Common Stock or a combination of cash and stock, as determined by the Company in its discretion.

The Company accounts for the Convertible Notes as a liability and equity component where the carrying value of the liability component will be valued based on a similar instrument. In accounting for the issuance of the Convertible Notes, the Company separated the Convertible Notes into liability and equity components. The equity component is not re-measured as long as it continues to meet the conditions for equity classification.

In accounting for the issuance of the Convertible Notes, the Company separated the Convertible Notes into liability and equity components based on their relative values. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated convertible feature. The carrying amount of the equity component, representing the conversion option, was determined by deducting the fair value of the liability component from the par value of the Convertible Notes. The difference between the principal amount of the Convertible Notes and the liability component represents the debt discount, which is recorded as a direct deduction from the related debt liability in the Consolidated Balance Sheets and amortized to interest expense using the effective interest method over the seven-year term of the Convertible Notes. The equity component of the Convertible Notes of approximately $88.3 million is included in additional paid-in capital in the Consolidated Balance Sheets and is not remeasured as long as it continues to meet the conditions for equity classification. Additionally, the Company recorded a deferred tax liability of $29.8 million in relation to the Convertible Notes.

The Company incurred transaction costs of approximately $7.5 million, including approximately $6.9 million that was paid from the gross proceeds of the Convertible Notes offering. In accounting for the transaction costs, the Company allocated the total amount incurred to the liability and equity components using the same proportions as the proceeds from the Convertible Notes. Transaction costs attributable to the liability component were recorded as a direct deduction from the related debt liability in the Consolidated Balance Sheets and amortized to interest expense over the seven-year term of the Convertible Notes. Transaction costs attributable to the equity component were netted with the equity component in additional-paid-in-capital.

The Convertible Notes consist of the following (in thousands): as of March 31, 2017 and December 31, 2016:

Liability component	**March 31, 2017**	**December 31, 2016**
Principal	$ 250,000	$ 250,000
Less: debt discount (1)	(88,546)	(90,807)
Less: deferred financing(1)	(4,595)	(4,729)
Net carrying value of the debt	$ 156,859	$ 154,464

(1) Included in the Consolidated Balance Sheets within Convertible Senior Notes (due 2023) and amortized to interest expense over the remaining life of the Convertible Senior Notes using the effective interest rate method.

The fair value of the debt at March 31, 2017 was approximately $336.9 million.

The following table sets forth total interest expense recognized related to the Convertible Notes for the three months ended March 31, 2017:

Components		(In thousands)
Contractual interest expense	$	1,887
Amortization of deferred financing		142
Amortization of debt discount		2,261
Total	$	4,290
Effective interest rate of the liability component		10.85%

The Capped Call Confirmations of $13.5 million are expected generally to reduce the potential dilution to the Common Stock upon any conversion of the Convertible Notes and/or offset the cash payments the Company is required to make in excess of the principal amount upon conversion of the Notes in the event that the market price of the Common Stock is greater than the strike price of the Capped Call Confirmations (which initially corresponds to the initial conversion price of the Convertible Notes and is subject to certain adjustments under the terms of the Capped Call Confirmations), with such reduction and/or offset subject to a cap based on the cap price of the Capped Call Confirmations. The Capped Call Confirmations have an initial cap price of $7.20 per share, which represents a premium of approximately 50% over the closing price of the Company's Common Stock on the NASDAQ Global Market on December 15, 2016, and is subject to certain adjustments under the terms of the Capped Call Confirmations. The Capped Call Confirmations will cover, subject to anti-dilution adjustments substantially similar to those applicable to the Convertible Notes, the number of shares of Common Stock that will underlie the Convertible Notes. The Capped Call Confirmations do not meet the criteria for separate accounting as a derivative as they are indexed to the Company's Common Stock. The premiums paid for the Capped Call Confirmations have been included as a net reduction to additional paid-in capital.

Note 8. Stockholders' Equity

Common Stock and Warrants

As of March 31, 2017, the Company was authorized to issue 250 million shares of Common Stock. Dividends on Common Stock will be paid when, and if, declared by the board of directors. Each stockholder is entitled to vote on all matters that are appropriate for stockholder voting and is entitled to one vote for each share held.

As discussed in "—Note 7. Debt Instruments", on December 21, 2016, the Company issued $250 million aggregate principal amount of Convertible Notes in a private offering. The Notes will mature on December 15, 2023, unless earlier repurchased, redeemed, or converted in accordance with their terms. The Notes are convertible at the option of the holders, under certain circumstances and during certain periods, into cash, shares of the Company's Common Stock or a combination thereof. Prior to the close of business on the business day immediately preceding September 15, 2023, the Notes are convertible at the option of the holders of the Notes only under certain conditions. On or after September 15, 2023, until the close of business on the second business day immediately preceding the maturity date, holders of the Notes may convert their Notes at their option at the conversion rate then in effect, irrespective of these conditions. The Company will settle conversions of the Notes by paying or delivering, as the case may be, cash, shares of Common Stock, or a combination of cash and shares of Common Stock, at the Company's election. The conversion rate will initially be 163.3987 shares of Common Stock per $1,000 principal amount of Notes (equivalent to an initial conversion price of approximately $6.12 per share of Common Stock). The conversion rate is subject to customary adjustments upon the occurrence of certain events.

Equity Incentive Plan

The Company's Equity Incentive Plans consist of the Amended and Restated 2007 Equity Incentive Plan (the "Plan") and the 2007 Director Option Plan (the "2007 Director Plan"). The Plan provides for the granting of restricted stock and options to purchase common stock in the Company to employees, advisors and consultants at a price to be determined by the Company's board of directors. The Plan is intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company's business. The 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. The Board of Directors, or its committee, is responsible for determining the individuals to be granted options, the number of options each individual will receive, the option price per share, and the exercise period of each option.

Stock Option Grants

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

	Three Months Ended March 31, 2017	Three Months Ended March 31, 2016
Expected stock price volatility	83.2%	81.2%
Risk free interest rate	2.1%	1.8%
Expected life of options (years)	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00

A summary of the Company's stock options for the three months ended March 31, 2017 is as follows:

	Number of Shares (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value (in millions)
Options outstanding, December 31, 2016	15,497.5	$ 7.37		
Granted	2,605.3	$ 5.19		
Exercised	(88.4)	$ 3.00		
Forfeited	(192.6)	$ 9.24		
Options outstanding, March 31, 2017	17,821.8	$ 7.05	7.5 years	$ 26.1
Vested and unvested expected to vest, March 31, 2017	16,637.6	$ 7.05	7.3 years	$ 24.8
Exercisable at March 31, 2017	8,564.0	$ 6.80	5.9 years	$ 15.7

As of March 31, 2017, the total unrecognized compensation cost related to non-vested stock options granted was $37.2 million and is expected to be recognized over a weighted average period of 2.8 years.

Restricted Stock Units ("RSUs") and Performance-Based Restricted Stock Units

RSUs awarded under the Plan are generally subject to graded vesting and are contingent on an employee's continued service. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses 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.

A summary of non-vested RSU activity under the Plan for the three months ended March 31, 2017 is as follows:

	Number of Share (in thousands)	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value (in millions)
Non-vested units as of December 31, 2016	744.4	$ 7.86		
Granted	2,308.1	$ 5.64		
Vested	(4.7)	$ 14.08		
Forfeited	(25.9)	$ 5.81		
Non-vested units as of March 31, 2017	3,021.9	$ 6.17	3	$ 21.5

On December 30, 2016, the Compensation Committee approved a form of Performance-Based Restricted Stock Unit Award Agreement (the “Performance-Based RSU Agreement”), to be used for performance-based RSUs granted to participants under the Amended and Restated Amicus Therapeutics, Inc. 2007 Equity Incentive Plan, including named executive officers. Certain awards under the form of Performance-Based RSU Agreement were granted in January 2017. The table above includes 401,413 market performance-based restricted stock units (“MPRSUs”) granted to executives. Vesting of these awards is contingent upon the Company meeting certain total shareholder return (TSR) levels as compared to a select peer group over the next three years. The MPRSUs cliff vest at the end of the three-year period and have a maximum potential to vest at 200% (802,826 shares) based on TSR performance. The related share-based compensation expense is determined based on the estimated fair value of the underlying shares on the date of grant and is recognized straight-line over the vesting term. The estimated fair value per share of the MPRSUs was $8.08 and was calculated using a Monte Carlo simulation model. The table above also includes 401,413 performance based awards that will vest over the next three years based on the Company achieving certain clinical milestones.

For the three months ended March 31, 2017, 4,680 of the RSUs vested and all non-vested units are expected to vest over their normal term. As of March 31, 2017, there was $13.4 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 3 years.

Compensation Expense Related to Equity Awards

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

	Three Months Ended March 31,	
	2017	**2016**
Equity compensation expense recognized in:		
Research and development expense	$ 2,753	$ 1,936
Selling, general and administrative expense	3,277	2,347
Total equity compensation expense	$ 6,030	$ 4,283

Note 9. Assets and Liabilities Measured at Fair Value

The Company’s financial assets and liabilities are measured at fair value and classified within the fair value hierarchy, which is defined as follows:

Level 1 — Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2 — Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

Level 3 — Inputs that are unobservable for the asset or liability.

A summary of the fair value of the Company's recurring assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2017 are identified in the following table (in thousands):

	Level 2	Total
Assets:		
Commercial paper	$ 110,659	$ 110,659
Corporate debt securities	124,028	124,028
Money market funds	2,197	2,197
	$ 236,884	$ 236,884

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$ —	$ 274,300	$ 274,300
Derivative liability	101	—	101
Deferred compensation plan liability	1,847	—	1,847
	$ 1,948	$ 274,300	$ 276,248

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

	Level 2	Total
Assets:		
Commercial paper	$ 68,390	$ 68,390
Corporate debt securities	74,535	74,535
Money market funds	1,829	1,829
	$ 144,754	$ 144,754

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$ —	$ 269,722	$ 269,722
Derivative liability	265	—	265
Deferred compensation plan liability	1,479	—	1,479
	$ 1,744	$ 269,722	$ 271,466

See "—Note 7. Debt Instruments" for the carrying amount and estimated fair value of the Company's Convertible Notes due in 2023, that falls into Level 2 category within the fair value level hierarchy. The fair value was determined using broker quotes in a non-active market for valuation.

The Company did not have any Level 3 assets as of March 31, 2017 or as of December 31, 2016.

Cash, Money Market Funds and Marketable Securities

The Company classifies its cash and money market funds within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available for sale and classifies these assets within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the three months ended March 31, 2017. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 2017.

Contingent Consideration Payable

The contingent consideration payable resulted from the acquisitions of Scioderm and Callidus. The most recent valuation was determined using a probability weighted discounted cash flow valuation approach. Using this approach, expected future cash flows are calculated over the expected life of the agreement, are discounted, and then exercise scenario probabilities are applied. The valuation is performed quarterly. Gains and losses are included in the statement of operations.

As discussed in "—Note 3. Acquisitions," on July 5, 2016, the Company entered into the MiaMed Agreement with MiaMed. MiaMed is a pre-clinical biotechnology company focused on developing protein replacement for CDKL5 and related diseases. Under the terms of the MiaMed Agreement, the Company agreed to pay up to an additional $83.0 million in connection with the achievement of certain clinical, regulatory and commercial milestones, for a potential aggregate deal value of $89.5 million. The MiaMed Agreement was accounted for as an asset acquisition and as such the Company determined that a liability for future milestone payments is not required to be recorded until the actual contingencies are met and will be recorded to research and development expenses when the contingency is resolved.

The contingent consideration payable for Scioderm and Callidus has been classified as a Level 3 recurring liability as its valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approach the estimated fair value could be significantly higher or lower than the fair value the Company determined. The Company may be required to record losses in future periods, including expenses related to CDKL5.

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

Contingent Consideration Liability	Fair value as of March 31, 2017	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	$ 237.8 million	Probability weighted discounted cash flow	Discount rate	0.8%-1.3%
			Probability of achievement of milestones	66.5% -100.0%
			Projected year of payments	2017-2019
Revenue-based milestones	$ 26.2 million	Monte Carlo	Revenue volatility	51%
			Probability of achievement of milestones	66.5%
			Discount rate	1.4%-2.7%
			Projected year of payments	2019-2035

The following significant unobservable inputs were used in the valuation of the contingent consideration payable of Callidus for the ATB200 Pompe program:

Contingent Consideration Liability	Fair value as of March 31, 2017	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	$ 9.9 million	Probability weighted discounted cash flow	Discount rate	13.0%
			Probability of achievement of milestones	32.0%-45.0%
			Projected year of payments	2018-2022

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

The following table shows the change in the balance of contingent consideration payable for the three months ended March 31, 2017 and 2016, respectively (in thousands):

	Three Months Ended March 31,	
	2017	2016
Balance, beginning of the period	$ 269,722	$ 274,077
Unrealized change in fair value change during the period, included in Statement of Operations	4,578	3,152
Balance, end of the period	$ 274,300	$ 277,229

Deferred Compensation Plan- Investment and Liability

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 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. The Company considers its investments in marketable securities, as available-for-sale and classifies these assets and related liability within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities.

Foreign Currency Exchange Rate Exposure

The Company transacts business in various foreign countries and therefore, is subject to risk of foreign currency exchange rate fluctuations. As such, in June 2016, the Company entered into a forward contract to economically hedge transactional exposure associated with commitments arising from trade accounts payable denominated in a currency other than the functional currency of the respective operating entity. The Company did not designate this forward contract as a hedging instrument under applicable accounting guidance and, therefore, the change in fair value resulted in a gain of $0.2 million that was recorded in the Consolidated Statements of Operations for the three months ended March 31, 2017 and the corresponding liability of $0.1 million was recorded as other current liability in the Consolidated Balance Sheets. The forward contract settles in monthly installments with the final installment settlement in June 2017.

Note 10. Basic and Diluted Net Loss per Common Share

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss attributable to common stockholders per common share (in thousands except share amounts):

	Three Months Ended March 31,	
Historical	**2017**	**2016**
Numerator:		
Net loss attributable to common stockholders	$ (54,992)	$ (43,691)
Denominator:		
Weighted average common shares outstanding — basic and diluted	142,770,629	125,178,517

Dilutive Common Stock equivalents would include the dilutive effect of Common Stock options, convertible debt units, RSUs 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):

	Three Months Ended March 31,	
	2017	**2016**
Options to purchase common stock	17,822	14,102
Convertible debt	40,850	—
Outstanding warrants, convertible to common stock	3,110	1,850
Unvested restricted stock units	3,022	479
Vested restricted stock units, unissued	50	—
Total number of potentially issuable shares	64,854	16,431

Note 11. Commitments and Contingencies

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company’s Common Stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. On May 26, 2016, the Court consolidated these lawsuits into a single action and appointed a lead plaintiff. The lead plaintiff filed a Consolidated Amended Class Action Complaint on July 11, 2016. On August 25, 2016, the defendants filed a motion to dismiss in response to the Consolidated Amended Class Action Complaint. This motion to dismiss was fully briefed on October 28, 2016. Lead plaintiff and defendants have reached an agreement in principal to fully and finally settle all claims asserted in the Consolidated Amended Class Action Complaint. The settlement is immaterial to the Company’s consolidated financial statements and is subject to court approval. The settlement amount is expected to be fully covered under insurance.

On or about March 3, 2016, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus’ behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against various officers and directors of the Company. Amicus itself is named as a nominal defendant. The derivative lawsuit alleges similar facts and circumstances as the three purported securities class action lawsuits described above and further alleges claims for breach of state law fiduciary duties, waste of corporate assets, unjust enrichment, abuse of control, and gross mismanagement based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. The plaintiff seeks, among other things, to require the Amicus Board to take certain actions to reform its corporate governance procedures, including greater shareholder input and a provision to permit shareholders to nominate candidates for election to the Board, along with restitution, costs of suit and attorney's fees. On February 7, 2017, the complaint was dismissed by the Court without prejudice.

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

Note 12. Subsequent Events

In April 2017, the Company paid $10 million for the event based milestone related to the achievement of 100% enrollment in the Phase 3 clinical study of SD-101.

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

Overview

We are a global patient-focused biotechnology company engaged in the discovery, development and commercialization of a diverse set of novel treatments for patients living with devastating rare and orphan diseases. Our lead product, migalastat HCl is a small molecule that can be used as a monotherapy and in combination with enzyme replacement therapy ("ERT") for Fabry disease.

Also in the pipeline, SD-101 is a product candidate in late-stage development, as a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa ("EB"). We are also leveraging our Chaperone-Advanced Replacement Therapy ("CHART™") platform technologies to develop novel ERT products for Pompe disease, Fabry disease, and potentially other lysosomal storage disorders ("LSDs"). We are also investigating preclinical and discovery programs in other rare and devastating diseases including cyclin-dependent kinase-like 5 ("CDKL5") deficiency. We believe that our platform technologies and our product pipeline uniquely position us at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

Our Strategy

Our strategy is to internally develop or acquire first-in-class or potentially best-in-class therapies that have the potential to provide significant benefits for individuals living with rare and devastating diseases. We intend to leverage our global capabilities to develop and commercialize our robust pipeline. During 2016, we made significant progress toward fulfilling our vision to build a leading global biotechnology company focused on rare and devastating diseases:

- *Global capabilities* . We have established a world-class international commercial infrastructure, with key leadership in place to execute the international launch of migalastat HCl that is currently underway.
- *Commercial success.* We received full approval in the European Union ("EU") of Galafold ("migalastat HCl") and commenced the commercial launch in Germany on May 30, 2016. We have achieved success with reimbursement in several EU member states and through our expanded access programs ("EAPs") and we continue to advance additional regulatory submissions worldwide.
- *Pompe clinical study.* We have reported positive preliminary data from a clinical study to evaluate Pompe disease patients treated with our novel treatment paradigm ATB200/AT2221.
- *Late-stage product development.* We continue to investigate SD-101, a proprietary topical medicine for all major types of EB, in a single Phase 3 registration study that we hope will support global applications for approval in a number of countries. SD-101 has been granted Breakthrough Therapy designation by the U.S. Food and Drug Administration ("FDA").
- *Patient-centricity.* We continue to focus on our patient advocacy, which has always been a critical component of the values of our corporate culture, throughout all levels of the organization. The needs of patients in the rare disease community are at the center of our inventive science, our commercial organization, and our clinical programs.

Our Commercial Product and Product Candidates

Migalastat for Fabry Disease

Our Fabry franchise strategy is to develop migalastat HCl (which we may refer to as "migalastat") for all patients with Fabry disease as a monotherapy for patients with amenable mutations and in combination with ERT for all other patients. Migalastat was approved for use in the EU in May 2016 under the brand name Galafold™ as a first-line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. Outside of the EU and Switzerland, migalastat is an investigational product.

We have launched Galafold in several European markets, including countries such as Germany, UK, Italy and Switzerland on a commercial basis as well as in select other European markets through reimbursed EAPs and recognized net product sales of $4.2 million in the three months ended March 31, 2017 as compared to $2.8 million in the fourth quarter of 2016. We are currently pursuing the country-by-country pricing and reimbursement process in the EU member states and we have regulatory submissions under review in additional territories including countries such as Canada and Australia. We are also targeting a first half 2017 regulatory submission (Japanese New Drug Application, or J-NDA) in Japan.

For patients with non-amenable mutations, we are leveraging our CHART™ technology and advanced biologics capabilities to develop a proprietary Fabry ERT for co-formulation with migalastat. Master cell banking has been completed and process development work has commenced. Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations, and at this time, it is not intended for concomitant use with ERT.

SD-101 for EB

We are in Phase 3 development of a novel, late-stage, proprietary topical medicine, SD-101, a potentially first-to-market therapy for the treatment of skin blistering and lesions associated with all major types of EB. ESSENCE, a Phase 3 registration-directed study, was initiated in March of 2015 and completed enrollment of more than 160 patients in April of 2017. ESSENCE is a randomized, double-blind, placebo-controlled study being conducted at multiple sites worldwide that is designed to evaluate the safety and efficacy of SD-101 6% in patients with any of the three major types of EB, who are at least one-month old. Participants are being randomized 1:1 to two treatment groups receiving either SD-101 6% or placebo applied over their entire body once daily for three months.

SD-101 was one of the first therapies to receive Breakthrough Therapy designation by the FDA in 2013, following the completion of the Phase 2a initial human proof-of-concept study. The FDA and EMA each have also reviewed the Phase 2b study results. Based on the currently available data and communications with FDA and EMA, we believe that the global regulatory pathway forward for SD-101 will require a single Phase 3 registration-directed study. The FDA agreed to a rolling NDA in the U.S., which was initiated in the fourth quarter of 2015. Following the Phase 2b study, our Paediatric Committee of the EMA has issued a positive opinion on our Paediatric Investigation Plan (“PIP”) for SD-101. A PIP is part of the EMA approval process and must be accepted prior to a submission of an MAA in the EU. Results from the Phase 3 study are anticipated in mid-2017 to support marketing applications for SD-101 in the U.S., EU, and other regions.

Novel ERT for Pompe Disease

We are leveraging our biologics capabilities and CHART™ platform to develop a novel treatment paradigm, ATB200/AT2221, for Pompe disease. This ERT consists of a uniquely engineered recombinant human acid alpha-glucosidase (“rhGAA”) enzyme, ATB200, with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone (AT2221) to improve activity and stability. We acquired ATB200 as well as our enzyme targeting technology through our purchase of Callidus Biopharma.

The small molecule pharmacological chaperone AT2221 is not an active ingredient that contributes directly to GAA substrate reduction but instead acts to stabilize ATB200. AT2221 binds and stabilizes ATB200 in the circulation to improve the uptake of active enzyme into key disease-relevant tissues, resulting in increased clearance of accumulated substrate, glycogen. The novel combination has been patented for method of use, and ATB200, following significant manufacturing scale-up, is our first biologic to enter clinical development. In preclinical studies, administration of ATB200/AT221 resulted in greater tissue GAA enzyme levels and further substrate reduction compared to the currently approved ERT for Pompe disease (alglucosidase alfa).

A Phase 1/2 clinical study, ATB200-02, was initiated in December of 2015 to investigate our novel Pompe treatment paradigm in Pompe patients. The primary objective is to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 for an 18-week primary treatment period followed by a long-term extension. The three patient cohorts, enrolling up to ~20 total patients across all cohorts, are ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Reported preliminary data highlights show safety data for 13 patients through interim data analysis (maximum 36 weeks) as well as PK and PD (muscle biomarker and disease substrate biomarker) data for 10 patients (eight ERT-switch patients and two naïve patients). ATB200/AT2221 safety measures showed no serious adverse events (SAEs) with TEAEs that were generally mild and transient. To date, ATB200/AT2221 has shown no infusion-associated reactions following 150+ infusions. The clinical PK profile was consistent with previously reported preclinical data (n=10). Reductions were observed in biomarkers of muscle damage (creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in ambulatory switch patients (N=8, week 18) and naïve patients (N=2, week 4)). Reduction was also observed in a biomarker of glycogen substrate - Urine Hexose Tetrasaccharide (Hex4) - in all eight ERT-switch patients and both naïve patients.

CDKL5

We are researching a potential first-in-class protein replacement therapy approach for CDKL5 deficiency in preclinical studies. CDKL5 (cyclin-dependent kinase-like 5) is a gene on the X-chromosome encoding the CDKL5 protein that regulates the expression of several essential proteins for normal brain development. Genetic mutations in the CDKL5 gene result in CDKL5 protein deficiency and the disorder manifests clinically as persistent seizures starting in infancy, followed by severe impairment in neurological development. Most children affected by CDKL5 deficiency cannot walk or care for themselves and may also suffer from scoliosis, visual impairment, sensory issues, and gastrointestinal complications.

Potential Collaborations and Alliances

On a regular basis we consider potential collaborations, alliances, and other business development opportunities to enhance our strategic plan to develop and provide therapies to patients living with rare and orphan diseases and support our continued expansion as a commercial biotechnology company. We are currently exploring opportunities for licensing migalastat to further enhance the development of the product in markets around the world and we may consider opportunities for licensing our other product candidates in the future.

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 March 31, 2017 to the items that we disclosed as our significant accounting policies and estimates described in "—Note 2. Summary of Significant Accounting Policies" to the Company's financial statements as contained in the Company's Annual Report on Form 10-K, as amended, for the year ended December 31, 2016. 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.

Revenue Recognition

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when persuasive evidence of an arrangement exists, title to product and associated risk of loss has passed to the customer, which is typically upon receipt by the end customer, the price is fixed or determinable, collection of the amounts due are reasonably assured and the Company has no further performance obligations.

The Company's net product sales consist solely of sales of Galafold for the treatment of Fabry disease in the EU. The Company has recorded revenue on sales where Galafold is available either on a commercial basis or through a reimbursed early access program. Orders for Galafold are generally received from pharmacies and the ultimate payor is typically a government authority.

The Company records revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

Inventories and Cost of Goods Sold

Until regulatory approval of Galafold, the Company expensed all manufacturing costs of Galafold as research and development expense. Upon regulatory approval, the Company began capitalizing costs related to the purchase and manufacture of Galafold.

Inventories are stated at the lower of cost and net realizable value determined by the first-in, first-out method. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity as well as product shelf-life. In evaluating the recoverability of inventories produced, the probability that revenue will be obtained from the future sale of the related inventory is considered and inventory value is written down for inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statements of operations.

Cost of goods sold includes the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, and provisions for excess and obsolete inventory, as well as royalties payable. A portion of the inventory available for sale was expensed as research and development costs prior to regulatory approval and as such the cost of goods sold and related gross margins are not necessarily indicative of future cost of goods sold and gross margin.

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 March 31 2017		Three months ended March 31 2016	
Projects				
Third party direct project expenses				
Monotherapy Studies				
Migalastat (Fabry Disease — Phase 3)	$	2,850	$	3,947
SD-101 (EB-Epidermolysis Bullosa— Phase 3)		3,427		1,615
Combination Studies				
ATB200 + AT2221 (Pompe Disease — Phase 2)		8,932		5,790
Fabry CHART (Fabry Disease — Preclinical)		128		154
Total third party direct project expenses	$	15,337	$	11,506
Other project costs (1)				
Personnel costs		11,482		8,418
Other costs (2)		4,057		3,501
Total other project costs	$	15,539	$	11,919
Total research and development costs	$	30,876	$	23,425

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

Stock Option Grants

In accordance with the applicable guidance, we estimate the fair value of each equity award granted. 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 grant date fair 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. We will continue to use a blended weighted average approach using our own historical volatility and other similar public entity volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The average expected life was determined using a “simplified” method of estimating the expected exercise term which is the mid-point between the vesting date and the end of the contractual term. As our stock price volatility has been over 75% and we have experienced significant business transactions, we believe that we do not have sufficient reliable exercise data in order to justify a change from the use of the “simplified” method of estimating the expected exercise term of employee stock option grants. The risk-free interest rate is based on U.S. Treasury, zero-coupon issues with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on expected turnover as well as a historical analysis of actual option forfeitures.

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

	Three months ended March 31, 2017		Three months ended March 31, 2016	
Expected stock price volatility		83.2%		81.2%
Risk free interest rate		2.1%		1.8%
Expected life of options (years)		6.25		6.25
Expected annual dividend per share	$	0.00	$	0.00

Restricted Stock Units ("RSUs") and Performance-Based Restricted Stock Units

The RSUs awarded 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 our common stock, par value $0.01 per share ("Common Stock") underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

On December 30, 2016, the Compensation Committee approved a form of Performance-Based Restricted Stock Unit Award Agreement (the "Performance-Based RSU Agreement"), to be used for performance-based RSUs granted to participants under the Amended and Restated Amicus Therapeutics, Inc. 2007 Equity Incentive Plan, including named executive officers. Certain awards under the form of Performance-Based RSU Agreement were granted in January 2017. The table above includes 401,413 market performance-based restricted stock units ("MPRSUs") granted to executives. Vesting of these awards is contingent upon the Company meeting certain total shareholder return (TSR) levels as compared to a select peer group over the next three years. The MPRSUs cliff vest at the end of the three-year period and have a maximum potential to vest at 200% (802,826 shares) based on TSR performance. The related share-based compensation expense is determined based on the estimated fair value of the underlying shares on the date of grant and is recognized straight-line over the vesting term. The estimated fair value per share of the MPRSUs was $8.08 and was calculated using a Monte Carlo simulation model. The table above also includes 401,413 performance based awards that will vest over the next three years based on the Company achieving certain clinical milestones.

Results of Operations

Three months Ended March 31, 2017 versus March 31, 2016

Net Product Sales. Net product sales were $4.2 million for Galalfold for the three months ended March 31, 2017. Galalfold was approved for sale in the EU in May 2016 and has been launched in several European markets, including countries such as Germany, UK and Switzerland as well as in select other European markets through reimbursed EAPs.

Cost of Goods Sold. Cost of goods sold includes manufacturing costs as well as estimated royalties associated with sales of our product. Cost of goods sold as a percentage of net sales was 18.6% for the three months ended March 31, 2017.

Research and Development Expense. Research and development expense was $30.9 million during the three months ended March 31, 2017, representing an increase of $7.5 million or 32.1% from $23.4 million for the three months ended March 31, 2016. The increase in research and development costs were primarily due to manufacturing costs related to clinical products and increases in clinical research costs for the enrollment of clinical studies for: the Pompe program of $3.1 million and the EB program of $1.8 million. Other increases were in personnel costs of approximately $3.1 million.

Selling, General and Administrative Expense. Selling, general and administrative expense was $19.1 million for the three months ended March 31, 2017, representing an increase of $3.4 million or 21.7% from $15.7 million for the three months ended March 31, 2016. The increase was primarily due to efforts to support the international activities and commercial launch of Galafold as well as the increases in personnel costs of approximately $3.0 million.

Changes in Fair Value of Contingent Consideration Payable. For the three months ended March 31, 2017, we recorded loss of $4.6 million representing a change of $1.4 million from the $3.2 million of expense for the three months ended March 31, 2016. The change in the fair value resulted primarily from increase in the Scioderm contingent consideration of $4.0 million, and an increase in the Callidus contingent consideration of $0.6 million. The fair value is impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods and rates.

Depreciation Expense . Depreciation expense was $0.8 million for the three months ended March 31, 2017, representing an increase of $0.1 million as compared to $0.7 million for the three months ended March 31, 2016. Depreciation was higher due to increased asset acquisitions, resulting in a higher depreciation base in 2017.

Interest Income. Interest income was $0.8 million for the three months ended March 31, 2017, representing an increase of $0.5 million from $0.3 million for the three months ended March 31, 2016. 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 $4.3 million for three months ended March 31, 2017, representing an increase of $3.4 million from $0.9 million for the three months ended March 31, 2016. Interest expense was higher primarily due to the $250 million convertible debt offering in December 2016.

Other income/ expense. Other income for the three months ended March 31, 2017 was $0.6 million, as compared to expense of $0.1 million for the three months ended March 31, 2016. The change was primarily from gain on foreign exchange transactions.

Liquidity and Capital Resources

Source of Liquidity

As a result of our significant research and development expenditures as well as expenditures to build a commercial organization to support the launch of Galafold, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have historically funded our operations principally through the issuance and sale of stock, collaborations, debt financings, grants and non-refundable license fees.

Cash flows

As of March 31, 2017, we had cash and cash equivalents and marketable securities of $279.8 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. For more details on the cash, cash equivalents and marketable securities, refer to "—Note 5. Cash, Money Market Funds and Marketable Securities," in our Notes to Consolidated Financial Statements.

Net Cash Used in Operating Activities

Net cash used in operations for the three months ended March 31, 2017 was $49.8 million due primarily to the net loss for the three months ended March 31, 2017 of $55.0 million, which was offset by increase in prepaid expenses of $5.8 million and a decrease of $1.3 million in accounts payable and accrued expenses, mainly related to program expenses and support for the commercial launch of Galafold.

Net cash used in operations for the three months ended March 31, 2016 was $44.9 million, due primarily to the net loss for the three months ended March 31, 2016 of $43.7 million and also by a decrease in accounts payable and accrued expenses of $9.7 million and increase in deferred investment balances of $0.4 million.

Net Cash Used in Investing Activities

Net cash used in investing activities for the three months ended March 31, 2017 was $92.4 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash used in investing activities reflects $161.1 million for the purchase of marketable securities, $0.7 million for the acquisition of property and equipment, partially offset by $69.4 million for the sale and redemption of marketable securities.

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

Net Cash Used in Financing Activities

Net cash used in financing activities for the three months ended March 31, 2017 was $0.1 million. Net cash used in financing activities reflects $0.3 million from vesting of RSUs and $0.1 million in payments on the capital lease arrangements, partially offset by $0.3 million received from exercise of stock options.

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

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;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the significant cost of new ERT cell line development and manufacturing as well as the cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of LSDs;
- the future results of on-going or later clinical trials for SD-101, including our ability to obtain regulatory approvals and commercialize SD-101 and obtain market acceptance of SD-101;
- the future results of on-going preclinical and later clinical trials for CDKL5, including our ability to obtain regulatory approvals and commercialize CDKL5 and obtain market acceptance for CDKL5;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to obtain reimbursement for migalastat HCI;
- our ability to obtain market acceptance of migalastat HCl in the EU;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property-related claims;
- the extent to which we acquire or invest in businesses, products and technologies;
- our ability to successfully integrate our recent acquisitions of Scioderm and MiaMed and their products and technologies into our business, including the possibility that the expected benefits of the transactions 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.

While we generated revenue from product sales since the second half of 2016, in the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We may seek additional funding through public or private financings of debt or equity. We believe that our existing cash and cash equivalents and short-term investments will be sufficient to fund the current operating plan into the second half of 2018.

Financial Uncertainties Related to Potential Future Payments

Milestone Payments / Royalties

We acquired exclusive worldwide patent rights to develop and commercialize migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with MSSM. This agreement expires upon expiration of the last of the licensed patent rights, which will be in 2018 in the U.S. and 2019 in Europe and Japan for monotherapy. If we develop a product for combination therapy of specific pharmacological chaperone such as migalastat plus an ERT for certain Lysosomal Storage Disorders such as Fabry disease and a patent issues from the pending MSSM applications covering such a combination therapy(ies), expiration for the combination product(s) will be 2024.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, then we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For migalastat in the first quarter of 2017, we incurred $0.1 million of royalty expense under the agreement with MSSM.

In November 2013, we entered into the Revised Agreement with GlaxoSmithKline ("GSK"), pursuant to which we have obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the prior agreement entered into between us and GSK in July 2012. Under the terms of the Revised Agreement, there was no upfront payment from us to GSK. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones up to $40 million, as well as tiered royalties in the mid-teens in eight major markets outside the U.S. In addition, because we reacquired worldwide rights to migalastat, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement. In the first quarter of 2017, we recognized approximately $0.5 million of royalty expense under the agreement with GSK.

As part of the merger agreement with Scioderm, we have agreed to pay up to an additional $361 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain clinical and regulatory milestones, and $257 million to Scioderm stockholders, option holders, and warrant holders upon achievement of certain sales milestones. If SD-101 is approved, EB qualifies as a rare pediatric disease and we will request a Priority Review Voucher. If the Priority Review Voucher is obtained and subsequently sold, we will pay Scioderm stockholders, option holders and warrant holders the lesser of $100 million in the aggregate or 50% of the proceeds of such sale. In April 2016, while the total clinical and regulatory approval milestone payments remain unchanged at $361 million, the allocation between the clinical and regulatory approval milestone payments were revised as follows: clinical milestones of up to $81 million and regulatory approval milestones of up to $280 million. The commercial milestone payments of up to $257 million remained unchanged. During the second quarter of 2016, we reached the first event-based milestone for Scioderm, which was the 50% enrollment of patients in the Phase 3 study. The milestone payment for this event was $5.0 million, which was paid in cash during the second quarter of 2016. At the end of the first quarter of 2017, we achieved 100% enrollment in the Phase 3 clinical study and the milestone payment due for this event, $10 million was paid in April 2017.

As part of the acquisition of Callidus, we will be obligated to make additional payments to the former stockholders of Callidus upon the achievement by the Company of certain clinical milestones of up to $35 million and regulatory approval milestones of up to $105 million as set forth in the merger agreement, provided that the aggregate consideration shall not exceed $130 million. We may, at our election, satisfy certain milestone payments identified in the merger agreement aggregating $40 million in shares of our Common Stock (calculated based on a price per share equal to the average of the last closing bid price per share for the Common Stock on The NASDAQ Global Select Market for the ten trading days immediately preceding the date of payment). The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that we are permitted to, but choose not to, satisfy in Common Stock), as a result of the terms of the merger agreement, the rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash. During the second quarter of 2016, we reached the first clinical milestone for Callidus, which was the dosing of the first patient in a Phase 1 or 2 study. The milestone payment for this event was $6.0 million which was paid in the Company’s stock during the second quarter of 2016.

As part of the acquisition of MiaMed, we will be obligated to make additional payments to the former stockholders of MiaMed upon the achievement by the Company of certain clinical milestones of up to $8 million, regulatory approval milestones of up to $10 million, and commercial milestones up to $65 million. Any milestone payment may be satisfied in cash, shares of Common Stock, or a combination of both. The milestone payments not permitted to be satisfied in Common Stock (as well as any payments that we are permitted to, but choose not to, satisfy in Common Stock), as a result of the terms of the merger agreement, the rules of The NASDAQ Global Select Market, or otherwise, will be paid in cash. No milestone payments in connection with the acquisition of MiaMed have been paid.

Recent Accounting Pronouncements

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

ITEM 3. Quantitative and Qualitative Disclosures about Market Risk

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio. At March 31, 2017, we held $279.8 million in cash, cash equivalents and available for sale securities and due to the short-term maturities of our investments, we do not believe that a 10% change in average interest rates would have a significant impact on our interest income. At March 31, 2017, our cash, cash equivalents and available for sale securities were all due on demand or within one year. Our outstanding debt has a fixed interest rate and therefore, we have no exposure to interest rate fluctuations.

We have operated primarily in the U.S. with international operations increasing since the last quarter of 2015. We do conduct some clinical activities with vendors outside the U.S. While most expenses are paid in U.S. dollars, we now have increased transactions of expenses and cash flows in foreign currencies that are exposed to changes in foreign currency rates. Foreign currency forward contracts used to offset these exposures are not designated as hedges.

ITEM 4. CONTROLS AND PROCEDURES

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

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

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

Since October 1, 2015, three purported securities class action lawsuits have been commenced in the United States District Court for New Jersey, naming as defendants the Company, its Chairman and Chief Executive Officer, and in one of the actions, its Chief Medical Officer. The lawsuits allege violations of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company related to the regulatory approval path for migalastat. The plaintiffs seek, among other things, damages for purchasers of the Company's Common Stock during different periods, all of which fall between March 19, 2015 and October 1, 2015. It is possible that additional suits will be filed, or allegations received from stockholders, with respect to similar matters and also naming the Company and/or its officers and directors as defendants. On May 26, 2016, the Court consolidated these lawsuits into a single action and appointed a lead plaintiff. The lead plaintiff filed a Consolidated Amended Class Action Complaint on July 11, 2016. On August 25, 2016, the defendants filed a motion to dismiss in response to the Consolidated Amended Class Action Complaint. This motion to dismiss was fully briefed on October 28, 2016. Lead plaintiff and defendants have reached an agreement in principal to fully and finally settle all claims asserted in the Consolidated Amended Class Action Complaint. The settlement is immaterial to the Company's consolidated financial statements and is subject to court approval. The settlement amount is expected to be fully covered under insurance.

On or about March 3, 2016, a derivative lawsuit was filed by an Amicus shareholder purportedly on Amicus' behalf in the Superior Court of New Jersey, Middlesex County, Chancery Division, against various officers and directors of the Company. Amicus itself is named as a nominal defendant. The derivative lawsuit alleges similar facts and circumstances as the three purported securities class action lawsuits described above and further alleges claims for breach of state law fiduciary duties, waste of corporate assets, unjust enrichment, abuse of control, and gross mismanagement based on allegedly false and misleading statements made by Amicus related to the regulatory approval path for migalastat HCl. The plaintiff seeks, among other things, to require the Amicus Board to take certain actions to reform its corporate governance procedures, including greater shareholder input and a provision to permit shareholders to nominate candidates for election to the Board, along with restitution, costs of suit and attorney's fees. On February 7, 2017, the complaint was dismissed by the Court without prejudice.

These lawsuits and any other related lawsuits are subject to inherent uncertainties and the actual cost will depend upon many unknown factors. The outcome of the litigation is necessarily uncertain and we could be forced to expend significant resources in the defense of these suits, 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, 2016, as amended.

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

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

We did not repurchase any shares of our Common Stock during the three months ended March 31, 2017. We have not announced any plans or programs for the repurchase of our Common Stock. However, employees surrendered 50,950 shares to the Company, during the three months ended March 31, 2017 at a weighted average price of $5.13 per share for the payment of the minimum tax liability withholding obligations upon the vesting of RSUs. We do not consider this a share buyback program.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

Exhibit Number	Description
3.1(1)	Restated Certificate of Incorporation
3.2(2)	Certificate of Amendment to the Company's Restated Certificate of Incorporation, as amended
3.3 (3)	Amended and Restated By-laws
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101	The following financial information from this Quarterly Report on Form 10-Q for the three months ended March 31, 2017, formatted in XBRL (Extensible Business Reporting Language) and filed electronically herewith: (i) the Consolidated Balance Sheets; (ii) the Consolidated Statements of Operations; (iii) the Consolidated Statements of Comprehensive Loss; (iv) the Consolidated Statements of Cash Flows; (v) and the Notes to the Consolidated Financial Statements

(1) Incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on August 5, 2015.

(2) Incorporated by reference to Exhibit 3.2 to our Quarterly Report on Form 10-Q filed on August 5, 2015.

(3) Incorporated by reference to Exhibit 3.3 to our Registration Statement on Form S-1.

SIGNATURES

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

AMICUS THERAPEUTICS, INC.

Date: May 9, 2017	By:	/s/ John F. Crowley
		John F. Crowley Chairman and Chief Executive Officer (Principal Executive Officer)
Date: May 9, 2017	By:	/s/ William D. Baird III
		William D. Baird III Chief Financial Officer (Principal Financial Officer)

Exhibit Number	Description
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101	The following financial information from this Quarterly Report on Form 10-Q for the three months ended March 31, 2017, 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.

Exhibit 31.1

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

I, John F. Crowley, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: May 9, 2017

/s/ John F. Crowley

John F. Crowley
Chairman and Chief Executive Officer

Exhibit 31.2

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

I, William D. Baird III, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: May 9, 2017

/s/ William D. Baird III
William D. Baird III
Chief Financial Officer

Exhibit 32.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended March 31, 2017, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: May 9, 2017	By:	/s/ John F. Crowley
		John F. Crowley
		Chairman and Chief Executive Officer
Date: May 9, 2017	By:	/s/ William D. Baird III
		William D. Baird III
		Chief Financial Officer