AMICUS THERAPEUTICS INC
Form 10-Q
August 07, 2017
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2017

OR

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

For the transition period from to

Commission file number 001-33497

Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**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 x No o

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 x	Accelerated filer o
Non-accelerated filer o	Smaller reporting company o
(Do not check if a smaller reporting company)	Emerging growth company o

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. o

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

The number of shares outstanding of the registrant s common stock, $0.01 par value per share, as of July 25, 2017 was 164,566,069 shares.

Table of Contents

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended June 30, 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 .

Table of Contents

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 i 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;
- the future results of on-going or subsequent 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 subsequent 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 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 acquisition of Scioderm, Inc. and its 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.

Table of Contents

PART I. **FINANCIAL INFORMATION**

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

Amicus Therapeutics, Inc.

Consolidated Balance Sheets *(Unaudited)*

(in thousands, except share and per share amounts)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 37,394	$ 187,026
Investments in marketable securities	189,838	143,325
Accounts receivable	3,786	1,304
Inventories	3,948	3,416
Prepaid expenses and other current assets	6,023	4,993
Total current assets	240,989	340,064
Property and equipment, less accumulated depreciation of $13,951 and $12,495 at June 30, 2017 and December 31, 2016, respectively	10,471	9,816
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	3,009	2,468
Total Assets	$ 938,966	$ 1,036,845
Liabilities and Stockholders Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 35,645	$ 41,008
Deferred reimbursements, current portion	18,850	13,850
Contingent consideration payable, current portion	46,188	56,101
Total current liabilities	100,683	110,959
Deferred reimbursements	16,906	21,906
Convertible notes	159,171	154,464
Contingent consideration payable	219,162	213,621
Deferred income taxes	173,869	173,771
Other non-current liability	2,283	1,973
Commitments and contingencies		
Stockholders equity:		
Common stock, $0.01 par value, 250,000,000 shares authorized, 143,371,243 and 142,691,986 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	1,485	1,480
Additional paid-in capital	1,132,229	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax expense of $1,293 at June 30, 2017 and December 31, 2016	(192)	1,945
Unrealized gain on available-for securities	31	102

Warrants		16,076		16,076
Accumulated deficit		(882,737)		(779,608)
Total stockholders equity		266,892		360,151
Total Liabilities and Stockholders Equity	$	938,966	$	1,036,845

See accompanying notes to consolidated financial statements

Table of Contents

Amicus Therapeutics, Inc.

Consolidated Statements of Operations

(Unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Revenue:				
Net product sales	$ 7,158	$	$ 11,327	$
Cost of goods sold	1,061		1,836	
Gross Profit	6,097		9,491	
Operating Expenses:				
Research and development	31,985	18,281	62,861	41,706
Selling, general and administrative	19,311	19,300	38,443	35,001
Changes in fair value of contingent consideration payable	1,050	10,186	5,628	13,338
Restructuring charges		8		58
Depreciation	812	767	1,636	1,440
Total operating expenses	53,158	48,542	108,568	91,543
Loss from operations	(47,061)	(48,542)	(99,077)	(91,543)
Other income (expenses):				
Interest income	753	331	1,512	638
Interest expense	(4,179)	(1,055)	(8,469)	(2,000)
Other income (expense)	2,400	(2,237)	3,010	(2,289)
Loss before income tax (expense)/benefit	(48,087)	(51,503)	(103,024)	(95,194)
Income tax (expense)/ benefit	(49)	453	(105)	453
Net loss attributable to common stockholders	$ (48,136)	$ (51,050)	$ (103,129)	(94,741)
Net loss attributable to common stockholders per common share basic and diluted	$ (0.34)	$ (0.40)	$ (0.72)	(0.75)
Weighted-average common shares outstanding basic and diluted	143,000,718	129,122,175	142,886,614	127,160,943

See accompanying notes to consolidated financial statements

Table of Contents

Amicus Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss

(Unaudited)

(in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Net loss	$ (48,136)	$ (51,050)	$ (103,129)	(94,741)
Other comprehensive (loss)/ gain:				
Foreign currency translation adjustment (loss)/ gain	(1,678)	907	(2,136)	842
Unrealized (loss)/ gain on available-for-sale securities	(155)	87	(72)	316
Other comprehensive (loss)/ income	$ (1,833)	994	(2,208)	1,158
Comprehensive loss	$ (49,969)	$ (50,056)	$ (105,337)	$ (93,583)

See accompanying notes to consolidated financial statements

Table of Contents

Amicus Therapeutics, Inc.

Consolidated Statements of Cash Flows

(Unaudited)

(in thousands)

	Six Months Ended June 30,	
	2017	**2016**
Operating activities		
Net loss	$ (103,129)	$ (94,741)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	4,715	1,014
Depreciation	1,636	1,440
Stock-based compensation	11,567	8,748
Restructuring charges		58
(Gain)/ Loss on disposal of asset	(8)	17
Change in fair value of derivative liability	(265)	346
Non-cash changes in the fair value of contingent consideration payable	5,628	13,338
Foreign currency remeasurement loss	(3,003)	1,892
Non-cash income tax benefit		(453)
Non-cash deferred taxes	98	
Changes in operating assets and liabilities:		
Accounts receivable	(2,226)	
Inventories	(351)	(207)
Prepaid expenses and other current assets	(2,103)	(865)
Other non-current assets	(521)	(549)
Account payable and accrued expenses	(4,297)	(8,244)
Non-current liabilities	496	535
Net cash used in operating activities	(91,763)	(77,671)
Investing activities		
Sale and redemption of marketable securities	137,909	121,283
Purchases of marketable securities	(184,494)	(126,914)
Purchases of property and equipment	(2,279)	(4,608)
Net cash used in investing activities	(48,864)	(10,239)
Financing activities		
Proceeds from issuance of common stock, net of issuance costs		57,818
Proceeds from unsecured loan agreement		30,000
Payment of capital leases	(142)	(47)
Payment of contingent consideration	(10,000)	(5,000)
Purchase of vested restricted stock units	(1,003)	(657)
Proceeds from exercise of stock options	1,554	647
Payment of deferred financing fees	(28)	
Net cash used in/ (provided by) financing activities	(9,619)	82,761
Effect of exchange rate changes on cash and cash equivalents	614	(680)
Net decrease in cash and cash equivalents	**(149,632)**	**(5,829)**
Cash and cash equivalents at beginning of year/ period	187,026	69,485
Cash and cash equivalents at end of year/period	**$ 37,394**	**$ 63,656**
Supplemental disclosures of cash flow information		
Cash paid during the period for interest	$ 3,650	$ 276
Contingent consideration resolution in shares	$	$ 6,115
Capital expenditures unpaid at end of the period	$ 351	$

Capital expenditures funded by capital lease borrowings	$	$ 850

See accompanying notes to consolidated financial statements

Table of Contents

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 Company s 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. Migalastat was approved for use in the European Union (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. Additionally, based on a series of discussions with and written communication received from the U.S. Food and Drug Administration (the FDA), the FDA has informed the Company that it may now submit a New Drug Application (NDA) for migalastat. An additional Phase 3 study previously requested by the FDA to assess Gastrointestinal (GI) symptoms is no longer required before an NDA submission. The Company is preparing the NDA submission under Subpart H, which provides for accelerated approval and plan to submit an NDA to the FDA for migalastat for Fabry disease in the fourth quarter of 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). On May 31, 2017, SD-101 received Rare Pediatric Disease designation from the FDA. The FDA grants Rare Pediatric Disease designation for diseases that primarily affect children ages 18 years or younger and fewer than 200,000 persons in the U.S.

We are also developing ATB200/AT2221, a novel treatment paradigm for Pompe disease that consists of a unique recombinant enzyme co-administered with AT2221, a pharmacological chaperone. The Company may further leverage its Chaperone-Advanced Replacement Therapy (CHART) platform technologies to develop novel ERT products for 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 its platform technologies and its product pipeline uniquely position it at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases.

On July 12, 2017, the Company entered into an underwriting agreement (the Underwriting Agreement) with J.P. Morgan Securities LLC and Goldman Sachs & Co. LLC, as representatives of the several underwriters set forth on Schedule 1 thereto, relating to an underwritten public offering of the Company s common stock (the Offering). Under the terms of the Underwriting Agreement, the Company issued and sold 21,122,449 shares at a price to the public of $12.25 per share, resulting in gross proceeds of $258.8 million, before deducting underwriting discounts and commissions and estimated offering expenses payable by the Company. The

Offering closed on July 18, 2017 and the Company received net proceeds from the Offering, after deducting underwriting discounts and commissions and offering expenses payable by the Company of $243.2 million. See Note 12 Subsequent Events for more details.

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 2019.

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.

Table of Contents

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. The forward contract expired in June 2017.

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 June 30, 2017 have arisen from product sales 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 six months ended June 30, 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.

Table of Contents

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. Allowance as of June 30, 2017 are immaterial.

Inventories and Cost of Goods Sold

Prior to 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 material adverse adjustments to the Company s operating results.

Table of Contents

Recent Accounting Pronouncements

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features, (Part II) Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception*. Part I of this Update addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. When determining whether certain financial instruments should be classified as liabilities or equity instruments, a down round feature no longer precludes equity classification when assessing whether the instrument is indexed to an entity s own stock. Part II of this Update addresses the difficulty of navigating Topic 480, Distinguishing Liabilities from Equity, because of the existence of extensive pending content in the FASB Accounting Standards Codification®. For public business entities, the amendments in Part I of this Update are effective for fiscal years, and years, beginning after December 15, 2018. Early adoption is permitted for all entities, including adoption in an interim period. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

In May 2017, the FASB issued ASU 2017-09, *Compensation Stock Compensation (Topic 718): Scope of Modification Accounting*. The amendments provide guidance on determining which changes to the terms and conditions of share-based payment awards require an entity to apply modification accounting under Topic 718 *Compensation Stock Compensation.* An entity should account for the effects of a modification unless all the following are met: 1. The fair value (or calculated value or intrinsic value, if such an alternative measurement method is used) of the modified award is the same as the fair value (or calculated value or intrinsic value, if such an alternative measurement method is used) of the original award immediately before the original award is modified. If the modification does not affect any of the inputs to the valuation technique that the entity uses to value the award, the entity is not required to estimate the value immediately before and after the modification. 2. The vesting conditions of the modified award are the same as the vesting conditions of the original award immediately before the original award is modified. 3. The classification of the modified award as an equity instrument or a liability instrument is the same as the classification of the original award immediately before the original award is modified. The ASU is effective for all entities for annual periods, including interim periods within those annual periods, beginning after December 15, 2017. Early adoption is permitted, including adoption in any interim period. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

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.

Table of Contents

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 is currently assessing the impact that this standard will have 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 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. ASU 2014-09 also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. 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). ASU 2014-09 will become effective for the Company during the first quarter of 2018.

The Company continues to assess the impact of ASU 2014-09 on its business and financial statements and expects the adoption of ASU 2014-09 to have an impact to its financial reporting disclosures and internal controls over financial reporting (ICFR). The Company has developed implementation controls that allow the Company to properly and timely adopt the new revenue accounting standard on its effective date. The

Company will make continuous updates to the quarterly and year-end disclosures, with a focus on both status and internal controls over financial reporting.

The Company s implementation plan includes a phased implementation project plan, an understanding of the new standard and its requirements, assessment of the Company s revenue streams and specific contracts in the streams. Additionally, the Company continues to monitor modifications, clarifications and interpretations issued by the FASB that may impact its assessment. Upon completion of the Company s implementation plan and evaluation of the remaining revenue contracts, the Company plans to adopt additional internal controls over financial reporting for any new revenue arrangements. The Company is on target to complete its assessment of ASU 2014-09 and the impact on the Company s consolidated financial statements and related disclosures as of January 1, 2018. The Company has elected to adopt the new standard using the modified retrospective approach.

Table of Contents

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 Inc., (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 June 30, 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 remained unchanged at $361 million, the allocation between the clinical and regulatory approval milestone payments was 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. The Company determined the fair value of the contingent consideration to be $254.7 million at June 30, 2017, of which $46.2 million is payable in the next twelve months, resulting in an increase in the contingent consideration payable and related expense of approximately $0.7 million for the three months ended June 30, 2017. The expense is recorded in the Consolidated Statement of Operations as the change within fair value of contingent consideration payable.

Table of Contents

Acquisition of Callidus Biopharma, Inc.

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

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

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 six months ended June 30, 2017:

		(in millions)
Balance at December 31, 2016	$	197.8
Change in goodwill		
Balance at June 30, 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 six months ended June 30, 2017:

		(in millions)
Balance at December 31, 2016	$	486.7
Change in IPR&D		
Balance at June 30, 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 June 30, 2017, the Company held $37.4 million in cash and cash equivalents and $189.8 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 greater than 3 months but less than 1 year are classified as short-term, while investments that have maturities greater than 1 year are classified as long-term.

For the six months ended June 30, 2017, the Company recognized a gain of $0.2 million, related to derivative instruments not designated as hedging instruments in other expense in the Consolidated Statements of Operations. Because the forward contract expired in June 2017, there was no liability in the Consolidated Balance Sheets as of June 30, 2017.

Table of Contents

Cash and available-for-sale securities are all current unless mentioned otherwise and consisted of the following as of June 30, 2017 and December 31, 2016 (in thousands):

	As of June 30, 2017			
	Cost	Gross unrealized Gain	Gross unrealized Loss	Fair Value
Cash balances	$ 37,394	$	$	$ 37,394
Corporate debt securities, current portion	108,683	1	(35)	108,649
Commercial paper	80,724	65		80,789
Money market	350			350
Certificate of deposit	50			50
	$ 227,201	$ 66	$ (35)	$ 227,232
Included in cash and cash equivalents	$ 37,394	$	$	$ 37,394
Included in marketable securities	189,807	66	(35)	189,838
Total cash and marketable securities	$ 227,201	66	$ (35)	$ 227,232

	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 six months ended June 30, 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 June 30, 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 $93.7 million and $58.7 million as of June 30, 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.

15

Table of Contents

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 June 30, 2017 (in thousands):

	June 30, 2017	December 31, 2016
Work-in-process	$ 3,758	$ 3,308
Finished goods	190	108
Total inventories	$ 3,948	$ 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 nor have any inventory reserves been recorded to date.

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 (the Note Offering). 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, 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

Table of Contents

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.

The last reported sale price of the Company s Common Stock was equal to or more than 130% of the conversion price of the Convertible Notes for atleast 20 trading days of the 30 consecutive trading days ending on the last day of the second quarter. As a result, the Convertible Notes are currently convertible into the Company s Common stock as discussed above and at least until the end of the third quarter.

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 June 30, 2017 and December 31, 2016:

Liability component	June 30, 2017	December 31, 2016
Principal	$ 250,000	$ 250,000
Less: debt discount (1)	(86,314)	(90,807)
Less: deferred financing(1)	(4,515)	(4,729)
Net carrying value of the debt	$ 159,171	$ 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.

Table of Contents

The fair value of the debt at June 30, 2017 was approximately $445.9 million.

The following table sets forth total interest expense recognized related to the Convertible Notes for the three and six months ended June 30, 2017:

Components (In thousands)	Three Months ended June 30, 2017	Six Months ended June 30, 2017
Contractual interest expense	$ 1,867	$ 3,754
Amortization of deferred financing	80	222
Amortization of debt discount	2,232	4,493
Total	$ 4,179	$ 8,469

Effective interest rate of the liability component was 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 June 30, 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.

Table of Contents

Stock Option Grants

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Expected stock price volatility	82.7%	81.3%	83.2%	81.2%
Risk free interest rate	1.9%	1.3%	2.0%	1.7%
Expected life of options (years)	6.25	6.25	6.25	6.25
Expected annual dividend per share	$ 0.00	$ 0.00	$ 0.00	$ 0.00

A summary of the Company s stock options for the six months ended June 30, 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	2,889.5	$ 5.44		
Exercised	(464.4)	$ 3.82		
Forfeited	(648.0)	$ 11.06		
Options outstanding, June 30, 2017	17,274.6	$ 7.00	7.4 years	$ 60.6
Vested and unvested expected to vest, June 30, 2017	16,249.7	$ 7.00	7.3 years	$ 57.2
Exercisable at June 30, 2017	8,931.2	$ 6.69	6.2 years	$ 34.2

As of June 30, 2017, the total unrecognized compensation cost related to non-vested stock options granted was $34.7 million and is expected to be recognized over a weighted average period of 2.6 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 six months ended June 30, 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	2,348.7	$ 5.69		
Vested	(203.1)	$ 8.40		
Forfeited	(63.8)	$ 7.19		
Non-vested units as of June 30, 2017	2,826.2	$ 6.04	2.9 years	$ 28.5

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

Table of Contents

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 six months ended June 30, 2017, 203,106 of the RSUs vested and all non-vested units are expected to vest over their normal term. As of June 30, 2017, there was $12.4 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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Equity compensation expense recognized in:				
Research and development expense	$ 2,313	$ 1,966	$ 5,066	3,902
Selling, general and administrative expense	3,224	2,500	6,501	4,846
Total equity compensation expense	$ 5,537	$ 4,466	$ 11,567	8,748

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 June 30, 2017 are identified in the following table (in thousands):

	Level 2	Total
Assets:		
Commercial paper	$ 80,789	$ 80,789
Corporate debt securities	108,649	108,649
Money market funds	2,320	2,320
	$ 191,758	$ 191,758

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$	$ 265,350	$ 265,350
Derivative liability			
Deferred compensation plan liability	1,994		1,994
	$ 1,994	$ 265,350	$ 267,344

Table of Contents

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 June 30, 2017 or as of December 31, 2016.

Cash, Money Market Funds and Marketable Securities

The Company classifies its cash 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 and the money market funds 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 six months ended June 30, 2017. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the six months ended June 30, 2017.

Contingent Consideration Payable

The contingent consideration payable resulted from the acquisitions of Scioderm and Callidus Biopharma, Inc. (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.

Table of Contents

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 June 30, 2017	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	$228.1 million	Probability weighted discounted cash flow	Discount rate	1.0%-1.3%
			Probability of achievement of milestones	66.5% -70.0%
			Projected year of payments	2017-2019
Revenue-based milestones	$26.6 million	Monte Carlo	Revenue volatility	51%
			Probability of achievement of milestones	66.5%
			Discount rate	1.5%-2.4%
			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 June 30, 2017	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	$10.3 million	Probability weighted discounted cash flow	Discount rate	12.5%
			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.

22

Table of Contents

The following table shows the change in the balance of contingent consideration payable for the three and six months ended June 30, 2017 and 2016, respectively (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016