AMICUS THERAPEUTICS INC
Form 10-Q
November 05, 2018

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

ý QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2018
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)	(Zip Code)

(609) 662-2000
(Registrant's Telephone Number, Including Area Code)
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 ý No o
Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted 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 such files). Yes ý No o
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions 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
	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 ý

The number of shares outstanding of the registrant's common stock, $0.01 par value per share, as of October 29, 2018 was 189,289,881 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended September 30, 2018

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We have filed applications to register certain trademarks in the United States and abroad, including AMICUS THERAPEUTICS AND DESIGN, AMICUS ASSIST AND DESIGN, CHART AND DESIGN, AT THE FOREFRONT OF THERAPIES FOR RARE AND ORPHAN DISEASES, HEALING BEYOND DISEASE, OUR GOOD STUFF and Galafold® and design.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks, uncertainties and assumptions. Forward-looking statements are all statements, other than statements of historical facts, that discuss our current expectation and projections relating to our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management. These statements may be preceded by, followed by or include the words “aim,” “anticipate,” “believe,” “can,” “could,” “estimate,” “expect,” “forecast,” “intend,” “lik “outlook,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “will,” “would,” the negatives or plurals thereof and othe and terms of similar meaning, although not all forward-looking statements contain these identifying words.

We have based these forward-looking statements on our current expectations and projections about future events. Although we believe that our assumptions made in connection with the forward-looking statements are reasonable, we cannot assure you that the assumptions and expectations will prove to be correct. You should understand that the following important factors could affect our future results and could cause those results or other outcomes to differ materially from those expressed or implied in our forward-looking statements:

- the progress and results of our preclinical and clinical trials of our drug candidates;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the cost of manufacturing Pompe Enzyme Replacement Therapy (“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 preclinical research and subsequent clinical trials for cyclin-dependent kinase-like 5 (“CDKL5”) deficiency, including our ability to obtain regulatory approvals and commercialize CDKL5 therapies and obtain market acceptance for such therapies;
- 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 successfully commercialize migalastat HCl ;
- our ability to manufacture or supply sufficient clinical or commercial products;
- our ability to obtain reimbursement for migalastat HCl;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of migalastat HCl;
- our ability to obtain market acceptance of migalastat HCl;
- 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 acquired 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;
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators;
- fluctuations in foreign currency exchange rates; and
- changes in accounting standards.

In light of these risks and uncertainties, we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part I Item 1A — Risk

Factors of the Annual Report on Form 10-K for the fiscal year ended December 31, 2017, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Those factors and the other risk factors described therein are not necessarily all of the important factors that could cause actual results or developments to differ materially from those expressed in any of our forward-looking statements. Other unknown or unpredictable factors also could harm our results. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations or investments we may make. Consequently, there can be no assurance that actual results or developments anticipated by us will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, us. Given these uncertainties, investors are cautioned not to place undue reliance on such forward-looking statements.

You should read this Quarterly Report on Form 10-Q in conjunction with our Annual Report on Form 10-K for the fiscal year ended December 31, 2017 completely and with the understanding that our actual future results may be materially different from what we expect. These forward-looking statements speak only as of the date of this report. We undertake no obligation, and specifically decline any obligation, to publicly update or revise any forward-looking statements, even if experience or future developments make it clear that projected results expressed or implied in such statements will not be realized, except as may be required by law.

PART I. FINANCIAL INFORMATION

Item 1. Consolidated Financial Statements and Notes (unaudited)

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

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 201,827	$ 49,060
Investments in marketable securities	362,556	309,502
Accounts receivable	14,189	9,464
Inventories	6,311	4,623
Prepaid expenses and other current assets	16,151	19,316
Total current assets	601,034	391,965
Property and equipment, less accumulated depreciation of $15,483 and $12,515 at September 30, 2018 and December 31, 2017, respectively	10,659	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	6,099	5,200
Total Assets	$ 838,589	$ 627,024
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 54,330	$ 53,890
Deferred reimbursements	2,750	7,750
Contingent consideration payable	8,800	8,400
Total current liabilities	65,880	70,040
Deferred reimbursements	14,156	14,156
Convertible notes	172,186	164,167
Senior secured term loan	146,622	—
Contingent consideration payable	19,300	17,000
Deferred income taxes	6,465	6,465
Other non-current liabilities	3,029	2,346
Total liabilities	427,638	274,174
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 and 250,000,000 shares authorized, 189,254,341 and 166,989,790 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	1,941	1,721
Additional paid-in capital	1,731,174	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(875)	(1,659)
Unrealized gain on available-for-sale securities	(211)	(436)
Warrants	13,063	16,076
Accumulated deficit	(1,334,141)	(1,063,610)
Total stockholders' equity	410,951	352,850

Total Liabilities and Stockholders' Equity	$ 838,589	$ 627,024

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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue:				
Net product sales	$20,596	$10,874	$58,601	$22,201
Cost of goods sold	4,310	1,790	10,060	3,626
Gross profit	16,286	9,084	48,541	18,575
Operating expenses:				
Research and development	138,227	40,641	213,685	103,502
Selling, general and administrative	31,867	21,647	88,435	60,090
Changes in fair value of contingent consideration payable	1,300	(244,250)	2,700	(238,622)
Loss on impairment of assets	—	465,427	—	465,427
Depreciation	1,073	851	3,015	2,486
Total operating expenses	172,467	284,316	307,835	392,883
Loss from operations	(156,181)	(275,232)	(259,294)	(374,308)
Other income (expense):				
Interest income	2,721	1,190	7,371	2,702
Interest expense	(4,715)	(4,351)	(13,763)	(12,820)
Change in fair value of derivatives	—	—	(2,739)	163
Other (expense) income	(1,039)	2,044	(3,593)	4,891
Loss before income tax	(159,214)	(276,349)	(272,018)	(379,372)
Income tax benefit	51	164,683	1,104	164,578
Net loss attributable to common stockholders	$(159,163)	$(111,666)	$(270,914)	$(214,794)
Net loss attributable to common stockholders per common share — basic and diluted	$(0.84)	$(0.69)	$(1.47)	$(1.44)
Weighted-average common shares outstanding — basic and diluted	189,162,841	160,796,841	184,606,790	148,963,864

See accompanying notes to consolidated financial statements

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net loss	$(159,163)	$(111,666)	$(270,914)	$(214,794)
Other comprehensive (loss) gain:				
Foreign currency translation adjustment gain (loss), net of tax impact of $59, $0, $168, $0, respectively	665	(1,176)	1,167	(3,312)
Unrealized gain (loss) on available-for-sale securities	244	(131)	225	(203)
Other comprehensive income (loss)	$909	$(1,307)	$1,392	$(3,515)
Comprehensive loss	$(158,254)	$(112,973)	$(269,522)	$(218,309)

See accompanying notes to consolidated financial statements

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

	Nine Months Ended September 30,	
	2018	2017
Operating activities		
Net loss	$(270,914)	$(214,794)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of debt discount and deferred financing	7,868	7,179
Depreciation	3,015	2,486
Stock-based compensation	20,873	17,067
Loss on impairment	—	465,427
Gain on disposal of asset	—	(8)
Change in fair value of derivatives	2,739	(265)
Non-cash changes in the fair value of contingent consideration payable	2,700	(238,622)
Foreign currency remeasurement (gain)/ loss	775	(4,932)
Non-cash deferred taxes	—	(164,585)
Changes in operating assets and liabilities:		
Accounts receivable	(5,182)	(4,288)
Inventories	(2,049)	(3,386)
Prepaid expenses and other current assets	2,633	(3,358)
Other non-current assets	(1,101)	344
Account payable and accrued expenses	3,684	13,037
Non-current liabilities	681	637
Deferred reimbursement	(5,000)	(12,600)
Net cash used in operating activities	$(239,278)	$(140,661)
Investing activities		
Sale and redemption of marketable securities	388,135	230,981
Purchases of marketable securities	(440,963)	(450,358)
Capital expenditures	(4,571)	(3,398)
Net cash used in investing activities	$(57,399)	$(222,775)
Financing activities		
Proceeds from issuance of common stock, net of issuance costs	294,584	243,036
Proceeds from senior secured term loan, net of issuance costs	146,622	—
Payment of capital leases	(218)	(212)
Payment of contingent consideration	—	(10,000)
Purchase of vested restricted stock units	(2,681)	(1,067)
Proceeds from exercise of stock options	8,492	8,841
Payment of deferred financing fees	—	(28)
Proceeds from exercise of warrants	3,617	—
Net cash provided by financing activities	$450,416	$240,570
Effect of exchange rate changes on cash, cash equivalents and restricted cash	$(1,146)	$1,030
Net increase (decrease) in cash and cash equivalents and restricted cash	152,593	(121,836)
Cash and cash equivalents and restricted cash at beginning of period	$51,237	$187,413
Cash and cash equivalents and restricted cash at end of period	$203,830	$65,577
Supplemental disclosures of cash flow information		

Cash paid during the period for interest	$3,787	$3,662
Capital expenditures, unpaid	$538	$—
Capital expenditures funded by capital lease	$80	$—

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Notes to the Consolidated Financial Statements
(Unaudited)

Note 1. Description of Business

Amicus Therapeutics, Inc. (the “Company”) is a global patient-centric biotechnology company engaged in the discovery, development and commercialization of a diverse set of novel treatments for patients living with rare metabolic diseases. With one medicine for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease in the clinic and the recent addition of fourteen new gene therapy programs into the pipeline, including two clinical stage gene therapies for Batten disease, the Company has a leading portfolio of medicines for lysosomal storage disorders (“LSDs”).

The cornerstone of the Company’s portfolio is migalastat HCl (also referred to as “migalastat”), the first and only approved oral precision medicine for people living with Fabry disease who have amenable genetic variants. Migalastat is currently approved under the trade name Galafold® in the United States (“U.S.”), European Union (“EU”) and Japan, with additional approvals granted and applications pending in several other geographies. During the third quarter of 2018, the Company initiated the commercial launch of Galafold® in the U.S. for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant.

The lead biologics program of the Company’s pipeline is Amicus Therapeutics GAA (“AT-GAA”, also known as ATB200/AT2221), a novel, clinical-stage, potential best-in-class treatment paradigm for Pompe disease. The Company’s Chaperone-Advanced Replacement Therapy (“CHART®”) platform technology is leveraged to develop novel products for Pompe disease and potentially future other LSDs.

During the second half of 2018, the Company has expanded its portfolio to include fourteen new gene therapy programs. In September 2018, the Company acquired worldwide development and commercial rights for ten gene therapy programs for neurologic LSDs developed at The Center for Gene Therapy at The Research Institute at Nationwide Children’s Hospital and The Ohio State University through the acquisition of Celenex, Inc., (“Celenex”) a private, clinical stage gene therapy company, for cash consideration of $100.0 million and additional consideration payable upon the achievement of certain development and approval milestones. The acquisition establishes Amicus as a leading company in neurologic LSDs. The lead programs in CLN6, CLN3, and CLN8 Batten disease are potential first-to-market curative therapies for these rare, devastating diseases. For additional information see "—Note 3. Acquisitions."

In October 2018, the Company further expanded its gene therapy portfolio through a collaboration agreement with the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania (“Penn”) to pursue the research and development of novel gene therapies for four additional indications, including Pompe disease, Fabry disease, CDKL5 deficiency disorder (“CDD”) and one additional undisclosed rare metabolic disorder. This relationship will combine the Company's protein engineering and glycobiology expertise with Penn’s adeno associated virus (“AAV”) gene transfer technologies to develop AAV gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.
The Company believes that its platform technologies and its product pipeline uniquely positions it and drives its commitment to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

During the third quarter of 2018, the Company entered into a loan agreement with BioPharma Credit PLC, as the lender, for a $150.0 million non-dilutive senior secured term loan (the “Senior Secured Term Loan”) with an interest rate equal to 3-month LIBOR plus 7.50% per annum, subject to a floor and ceiling on the rate, which matures in five

years. The Company received net proceeds of $146.6 million in September 2018, after deducting fees and estimated expenses payable by the Company. There are no warrants or any equity conversion features associated with the Senior Secured Term Loan. The proceeds from this financing will be used to support the cost of the Celenex acquisition, its related development costs and other general corporate purposes. For additional information, see " —Note 6. Debt."

During the first quarter of 2018, the Company issued 20,239,839 shares of its common stock through an underwritten offering resulting in net proceeds of $294.6 million after deducting underwriting discounts and commissions and offering expenses payable by the Company. The Company expects to use the net proceeds of the offering for investment in the U.S. and international commercial infrastructure for migalastat HCl, investment in manufacturing capabilities for the ERT ATB200, the continued clinical development of its product candidates, research and development expenditures, clinical and pre-clinical trial expenditures, commercialization expenditures and for other general corporate purposes. For additional information, see “—Note 7. Stockholders’ Equity.”

The Company had an accumulated deficit of approximately $1.3 billion as of September 30, 2018 and anticipates incurring losses through the fiscal year ending December 31, 2018 and beyond. The Company has been able to fund its operating losses to date through stock offerings, debt issuances, payments from partners during the terms of the collaboration agreements, and other financing arrangements.

The current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe and gene therapy program operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact the Company's future capital requirements.

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 for the fiscal year ended December 31, 2017. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K for the fiscal year ended December 31, 2017.

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.

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.

Reclassification

Certain prior year amounts have been reclassified for comparative purposes. The reclassifications did not affect results of operations, net assets or cash flows.

Cash, Cash Equivalents, Restricted Cash and Marketable Securities

The Company considers all highly liquid investments purchased with a maturity of three months or less at the date of acquisition, to be cash equivalents. Marketable securities consist of fixed income investments with a maturity of greater than three months and other highly liquid investments that can be readily purchased or sold using established markets. These investments are classified as available-for-sale and are reported at fair value on the Company's consolidated balance sheet. Unrealized holding gains and losses are reported within comprehensive income (loss) in the statements of comprehensive loss. Fair value is based on available market information including quoted market prices, broker or dealer quotations or other observable inputs.

Restricted cash consists primarily of funds held to satisfy the requirements of certain agreements that are restricted in their use and is included in non-current assets on the Company's consolidated balance sheet.

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 Company's accounts receivable at September 30, 2018 have arisen from product sales primarily in the EU. 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.

Revenue Recognition

The Company's net product sales consist of sales of Galafold® for the treatment of Fabry disease primarily 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 ("EAP"). Orders for Galafold® are generally received from pharmacies and the ultimate payor is typically a government authority.

The Company recognizes revenue when its performance obligations to its customers have been satisfied, which occurs at a point in time when the pharmacies or distributors obtain control of Galafold®. The transaction price is determined based on fixed consideration in the Company's customer contracts and is recorded net of estimates for variable consideration, which are third party discounts and rebates. The identified variable consideration is recorded as a reduction of revenue at the time revenues from sales of Galafold® are recognized. The Company recognizes revenue to the extent that it is probable that a significant revenue reversal will not occur in a future period. These estimates may differ from actual consideration received. The Company evaluates these estimates each reporting period to reflect known changes.

The Company elected the portfolio approach practical expedient in applying ASC Topic 606 to its identified revenue streams. Contracts within each revenue stream have similar characteristics and the Company believes this approach would not differ materially from applying ASC Topic 606 to each individual contract.

Recent Accounting Developments - Guidance Adopted in 2018

ASU 2018-07 — In June 2018, the Financial Accounting Standards Board (the "FASB") issued Accounting Standards Update ("ASU") 2018-07, Compensation-Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting ("ASU 2018-07").These amendments expand the scope of Topic 718, Compensation—Stock Compensation (which currently only includes share-based payments to employees) to include share-based payments issued to non-employees for goods or services. Consequently, the accounting for share-based payments to non-employees and employees will be substantially aligned. ASU 2018-07 supersedes Subtopic 505-50,

Equity—Equity-Based Payments to Non-Employees, and is effective for all public entities for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years. Early adoption is permitted, but no earlier than a company's adoption date of Topic 606, Revenue from Contracts with Customers. The Company early adopted ASU 2018-07 in the second quarter of 2018 and there was no material impact on its consolidated financial statements from the adoption.

ASU 2018-02 — In February 2018, the FASB issued ASU 2018-02, Income Statement - Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income (“ASU 2018-02”). Prior to ASU 2018-02, U.S. GAAP required the remeasurement of deferred tax assets and liabilities as a result of a change in tax laws or rates to be presented in net income from continuing operations, even in situations in which the related income tax effects of items in accumulated other comprehensive income were originally recognized in other comprehensive income. As a result, such items, referred to as stranded tax effects, did not reflect the appropriate tax rate. Under ASU 2018-02, entities are permitted, but not required, to reclassify from accumulated other comprehensive income to retained earnings those stranded tax effects resulting from the Tax Cuts and Jobs Act of 2017. ASU 2018-02 is effective for all entities for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years. Early adoption is permitted. The Company adopted the new standard effective January 1, 2018. As a result of the adoption, the Company reclassified a gain of $383,000 from the foreign currency translation adjustment in accumulated other comprehensive loss to accumulated deficit in the consolidated balance sheet as of September 30, 2018.

ASU 2017-09 — In May 2017, the FASB issued ASU 2017-09, Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting (“ASU 2017-09”). The amendments in ASU 2017-09 provide guidance on determining which changes to the terms and conditions of share-based payment awards require an entity to apply modification accounting under ASU 2017-09. 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; and (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. ASU 2017-09 is effective for all entities for annual periods, including interim periods within those annual periods, beginning after December 15, 2017. The Company adopted this standard on January 1, 2018 and as the Company did not have any modification of awards, the adoption of the standard did not have a material impact on its consolidated financial statements.

ASU 2017-01 — In January 2017, the FASB issued ASU 2017-01, Business Combinations (Topic 805): Clarifying the Definition of a Business (“ASU 2017-01”). This ASU 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 ASU 2017-01 are effective for public companies for annual periods beginning after December 15, 2017, including interim periods within those periods. The amendments should be applied prospectively as of the beginning of the period of adoption. The Company adopted ASU 2017-01 on January 1, 2018. The adoption of the standard did not have a material impact on its consolidated financial statements. For additional information see "—Note 3. Acquisitions."

ASU 2016-18 — In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash (“ASU 2016-18”). The amendments of ASU 2016-18 require an entity to include amounts generally described as restricted cash and restricted cash equivalents with cash and cash equivalents when reconciling the beginning of period and end of period total amounts on the statement of cash flows. The amendments of ASU 2016-18 are effective for annual reporting periods beginning after December 15, 2017, and interim periods within those annual periods. The Company adopted the guidance in ASU 2016-18 effective January 1, 2018. In connection with the adoption of the standard, the Company applied the guidance retrospectively which resulted in an increase in cash flows from operations of $1.1 million on the statement of cash flows for the nine months ended September 30, 2017.

ASU 2016-16 — In October 2016, the FASB issued ASU 2016-16, Income Taxes (Topic 740): Intra-Entity Transfers of Assets Other Than Inventory ("ASU 2016-16"). ASU 2016-16 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 ASU 2016-16 are effective for public business entities for annual periods beginning after December 15, 2017, including interim reporting periods within those annual reporting periods. The Company adopted ASU 2016-16 on January 1, 2018 and there was no material impact from the adoption.

ASU 2016-01 — In January 2016, the FASB issued ASU No. 2016-01, Financial Instruments-Overall: Recognition and Measurement of Financial Assets and Financial Liabilities (“ASU 2016-01”). ASU 2016-01 changes accounting for equity investments, financial liabilities under the fair value option, and presentation and disclosure requirements for financial instruments. ASU 2016-01 does not apply to equity investments in consolidated subsidiaries or those accounted for under the equity method of accounting. In addition, the FASB clarified guidance related to the valuation allowance assessment when recognizing deferred tax assets resulting from unrealized losses on available-for-sale debt securities. Equity investments with readily determinable fair values will be measured at fair value with changes in fair value recognized in net income. Companies have the option to measure equity investments without readily determinable fair values at either fair value or at cost adjusted for changes in observable prices minus impairment. Companies that elect the fair value option for financial liabilities must recognize changes in fair value related to instrument-specific credit risk in other comprehensive income. Companies must assess valuation allowances for deferred tax assets related to available-for-sale debt securities in combination with their other deferred tax assets. ASU 2016-01 is effective beginning in the first quarter of 2018 and the Company adopted it in the first quarter of 2018. There was no impact on the Company’s consolidated financial statements and related disclosures upon adoption, as the Company does not have equity investments or liabilities with credit risk. In addition, the guidance relating to deferred tax assets did not result in a change in accounting treatment for the Company.

ASU 2014-09 — In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606) which along with amendments issued in 2015 and 2016, replaced substantially all current U.S. GAAP guidance on this topic and eliminated industry-specific guidance. The new revenue recognition standard requires entities to recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. Effective January 1, 2018, the Company adopted the new revenue recognition standard using the modified retrospective approach and applied this approach only to contracts that were not completed as of January 1, 2018. The timing of revenue recognition and treatment of contract costs remains unchanged under the new revenue recognition standard. As such, the adoption of the new revenue recognition standard did not have a material impact on the Company's consolidated financial statements. The information presented for the periods prior to January 1, 2018 has not been restated and is reported under the accounting standard in effect for those periods.

Recent Accounting Developments - Guidance Not Yet Adopted

In August 2018, the Securities Exchange Commission (“SEC”) issued Final Rule 33-10532, Disclosure Update and Simplification, which amends certain disclosure requirements that were redundant, duplicative, overlapping or superseded by other SEC disclosure requirements. The amendments generally eliminated or otherwise reduced certain disclosure requirements of various SEC rules and regulations. However, in some cases, the amendments require additional information to be disclosed, including changes in stockholders’ equity in interim periods. The rule is effective 30 days after its publication in the Federal Register. The rule was posted on October 4, 2018. On September 25, 2018, the SEC released guidance advising it will not object to a registrant adopting the requirement to include changes in stockholders’ equity in the Form 10-Q for the first quarter beginning after the effective date of the rule. The Company is currently assessing the impact that this standard will have on its consolidated financial statements upon adoption and expects to adopt the guidance in it's Form 10-Q for the period ended March 31, 2019.
ASU 2018-13 — In August 2018, the FASB issued ASU 2018-03, Fair Value Measurement (Topic 820): Disclosure Framework-Changes to the Disclosure Requirements for Fair Value Measurement (“ASU 2018-13”). The amendments modify the disclosure requirements in Topic 820. ASU 2018-13 is effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The amendments on changes in unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and the narrative description of measurement uncertainty should be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption. All other amendments should be applied retrospectively to all periods presented upon their effective date. Early adoption is permitted. An entity is

permitted to early adopt any removed or modified disclosures upon issuance of ASU 2018-13 and delay adoption of the additional disclosures until their effective date. The Company is currently assessing the impact that this standard will have on its consolidated financial statements upon adoption.

ASU 2017-12 — In August 2017, the FASB issued ASU 2017-12, Derivatives and Hedging (Topic 815): Targeted Improvements to Accounting for Hedging Activities ("ASU 2017-12"). The amendments in this ASU 2017-12 better align an entity's risk management activities and financial reporting for hedging relationships through changes to both the designation and measurement guidance for qualifying hedging relationships and the presentation of hedge results. To meet that objective, the amendments expand and refine hedge accounting for both nonfinancial and financial risk components and align the recognition and presentation of the effects of the hedging instrument and the hedged item in the financial statements. The amendments in ASU 2017-12 also make certain targeted improvements to simplify the application of hedge accounting guidance and ease the administrative burden of hedge documentation requirements and assessing hedge effectiveness. For public business entities, the amendment is effective for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years. Early adoption, including adoption in an interim period, is permitted. The Company is currently assessing the impact that this standard will have on its consolidated financial statements.

ASU 2017-11 — 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 ("ASU 2017-11"). Part I of ASU 2017-11 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 ASU 2017-11 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 ASU 2017-11 are effective for fiscal 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.

ASU 2017-08 — 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 ("ASU 2017-08"). The amendments in ASU 2017-08 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. ASU 2017-08 is effective for public business entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. 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.

ASU 2017-04 — In January 2017, the FASB issued ASU 2017-04, Intangibles - Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment ("ASU 2017-04"). 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.

ASU 2016-02 — In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) ("ASU 2016-02"). ASU 2016-02 requires the recognition of lease assets and lease liabilities on the balance sheet for all lease obligations and disclosing key information about leasing arrangements. ASU 2016-02 requires the recognition of lease assets and lease liabilities by lessees for those leases classified as operating leases under previous generally accepted accounting principles. ASU 2016-02 will be effective for the Company for all annual and interim periods beginning after December 15, 2018, including interim periods within those fiscal years. In August 2018, the FASB issued ASU 2018-11, Leases (Topic 842): Targeted Improvements, ("ASU 2018-11"). ASU 2018-11 provide entities with an additional transition method for adoption, whereby, an entity initially applies the new leases standard at the adoption date and recognizes a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. The Company will adopt these standards effective January 1, 2019, and expects to elect certain of the practical expedients permitted, including the expedient that permits the Company to retain its existing lease assessment and classification. The Company also expects to elect the transition method in ASU 2018-11. The Company is currently working through an adoption plan which includes the evaluation of lease contracts compared to the new standard. While the Company is currently assessing the impact that this standard will have on its consolidated financial statements, the Company expects to recognize lease liabilities and right of use assets.

Note 3. Acquisitions

In September 2018, the Company expanded its pipeline by acquiring the rights and related intellectual property of ten gene therapy programs through its acquisition of Celenex. Celenex is a private, clinical stage gene therapy company whose lead programs are ten gene therapy programs including CLN6 and CLN3, which are in clinical stage, and several programs in pre-clinical stage. Pursuant to the terms of the agreement, the Company acquired Celenex for cash consideration of $100 million. The Company has also agreed to pay up to an additional $15 million in connection with the achievement of certain development milestones, $262 million in connection with the achievement of certain regulatory approval milestones across multiple programs and up to $75 million in tiered sales milestone payments. Celenex has an exclusive license agreement with Nationwide Children's Hospital ("NCH"). Under this license agreement, NCH is eligible to receive development and sales based milestones of up to $7.8 million for each product.

The Company evaluated the Celenex transaction based on the guidance in Business Combinations (Topic 805) and concluded that the transaction did not meet the definition of a business and was an asset acquisition. Given the fact that the license has no alternative future use, the $100.0 million upfront payment was expensed to research and development expense in the Consolidated Statements of Operations for the three and nine months ended September 30, 2018 in accordance with ASC Topic 730, Research and Development.

Note 4. Cash, Cash Equivalents, Marketable Securities and Restricted Cash

As of September 30, 2018, the Company held $201.8 million in cash and cash equivalents and $362.6 million of available-for-sale debt 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 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.

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 three months but less than one year are classified as current, while investments that have maturities greater than one year are classified as long-term.

Cash, cash equivalents and marketable securities are classified as current unless mentioned otherwise below and consisted of the following:

	As of September 30, 2018			
(in thousands)	Cost	Gross unrealized Gain	Gross unrealized Loss	Fair Value
Cash and cash equivalents	$201,827	$ —	$ —	$201,827
Corporate debt securities, current portion	227,517	5	(123)	227,399
Commercial paper	82,704	—	(49)	82,655
Asset-backed securities	52,145	—	(44)	52,101
Money market	350	—	—	350
Certificates of deposit	51	—	—	51
	$564,594	$ 5	$ (216)	$564,383
Included in cash and cash equivalents	$201,827	$ —	$ —	$201,827
Included in marketable securities, current and non-current	362,767	5	(216)	362,556
Total cash, cash equivalents and marketable securities	$564,594	$ 5	$ (216)	$564,383

	As of December 31, 2017			
(in thousands)	Cost	Gross unrealized Gain	Gross unrealized Loss	Fair Value
Cash and cash equivalents	$49,060	$ —	$ —	$49,060
Corporate debt securities, current portion	199,314	1	(303)	199,012
Commercial paper	79,878	—	(75)	79,803
Asset-backed securities	30,346	—	(59)	30,287
Money market	350	—	—	350
Certificates of deposit	50	—	—	50
	$358,998	$ 1	$ (437)	$358,562
Included in cash and cash equivalents	$49,060	$ —	$ —	$49,060
Included in marketable securities	309,938	1	(437)	309,502
Total cash, cash equivalents and marketable securities	$358,998	$ 1	$ (437)	$358,562

For the nine months ended September 30, 2018 there were nominal realized gains. For the fiscal year ended December 31, 2017, 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 debt securities as of September 30, 2018 and December 31, 2017 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 debt securities in unrealized loss positions was $315.3 million and $295.1 million as of September 30, 2018 and December 31, 2017, respectively.

The following table provides a reconciliation of cash and cash equivalents, and restricted cash reported within the consolidated balance sheet that sum to the total of the same such amounts shown in the statement of cash flows.

(in thousands)	September 30, 2018	December 31, 2017	September 30, 2017	December 31, 2016
Cash and cash equivalents	$201,827	$ 49,060	$ 64,133	$187,026
Restricted cash	2,003	2,177	1,444	387

Cash and cash equivalents and restricted cash shown in the statement of cash flows	$ 203,830	$ 51,237	$ 65,577	$ 187,413

Note 5. Inventories

Inventories consist of raw materials, work-in-process and finished goods related to the manufacture of Galafold®. The following table summarizes the components of inventories:

(in thousands)	September 30, 2018	December 31, 2017
Raw materials	$ 2,954	$ 2,394
Work-in-process	$ 1,458	$ 1,449
Finished goods	1,899	780
Total inventories	$ 6,311	$ 4,623

Note 6. Debt

Senior Secured Term Loan due 2023

In September 2018, the Company entered into a loan agreement with BioPharma Credit PLC as the lender. The loan agreement provides for a $150 million senior secured term loan ("Senior Secured Term Loan") with an interest rate equal to the 3-month LIBOR plus 7.50% per annum and matures five years from the maturity date. The Senior Secured Term Loan will be repaid in four quarterly payments equal to 12.50% thereof starting on the forty-eight month anniversary of the date of the first credit extension with the balance due on the Maturity Date. Interest is payable quarterly in arrears. The Senior Secured Term Loan contains certain customary representations and warranties, affirmative and negative covenants and events of default applicable to the Company and certain of its subsidiaries, but does not include any financial covenants relating to the achievement or maintenance of revenue or cash flow. If an event of default occurs and is continuing, the lender may declare all amounts outstanding under the Senior Secured Term Loan to be immediately due and payable. The Company received net proceeds of $146.6 million in September 2018, after deducting fees and estimated expenses payable by the Company.

Convertible Notes due 2023

In December 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 Convertible 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 Convertible Notes will mature on December 15, 2023, unless earlier repurchased, redeemed, or converted in accordance with their terms. The Convertible 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. The net proceeds from the Note Offering were $243.0 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. 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 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 the Company's common stock, subject to adjustment under certain conditions. 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 at least 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 further described in "—Note 7. Stockholders' Equity," on February 15, 2018, the Company entered into an underwriting agreement relating to an underwritten public offering of 19,354,839 shares of the Company's common stock. Under the terms of the underwriting agreement, the Company granted the underwriters an option, exercisable for 30 days after February 16, 2018, to purchase up to an additional 2,903,225 shares of the Company's common stock, which was exercised with respect to 885,000 shares of the Company's common stock.

Subsequent to the underwritten public offering on February 15, 2018, the Company did not have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. As a result, the Company accounted for the portion of the bifurcated conversion feature and of the Capped Call Confirmations that would not be able to be net share settled as a current derivative liability and as a derivative asset, respectively. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations at February 15, 2018 was determined to be $507.4 million and $13.6 million, respectively, of which the portion that was determined to not be able to be net share settled was recorded with a corresponding impact to additional-paid-in-capital. Subsequent changes to fair value of the derivatives were recorded in the second quarter of 2018 through earnings on the Company's consolidated statements of operations resulting in a change in fair value of derivatives for the nine months ended September 30, 2018 of $2.7 million.

Following the approval by the stockholders of the Company on June 7, 2018, to increase the authorized shares of common stock to 500,000,000, the Company has sufficient unissued authorized shares to cover a conversion of the Convertible Notes. As a result, the derivative liability and derivative asset were reclassified into additional-paid-in-capital. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations at June 7, 2018 was determined to be $88.3 million and $2.4 million, respectively.

The Convertible Notes and Senior Secured Term Loan consist of the following (in thousands):

Liability component	September 30, 2018	December 31, 2017
Principal	$400,000	$250,000
Less: debt discount (1)	(76,946)	(81,566)
Less: deferred financing (1)	(4,246)	(4,267)
Net carrying value of the debt	$318,808	$164,167

(1) Included in the consolidated balance sheets within convertible notes and senior secured term loan and amortized to interest expense over the remaining life of the Convertible Notes and Senior Secured Term Loan using the effective interest rate method.

The following table sets forth total interest expense recognized related to the Convertible Notes and Senior Secured Term Loan for the three and nine months ended September 30, 2018, respectively:

Components (In thousands)	Three Months Ended September 30, 2018	Nine Months Ended September 30, 2018
Contractual interest expense	$ 1,969	$ 5,744
Amortization of debt discount	2,609	7,620
Amortization of deferred financing	137	399
Total	$ 4,715	$ 13,763

Note 7. Stockholders' Equity

Common Stock and Warrants

On February 15, 2018, the Company announced the pricing of an underwritten offering of 19,354,839 shares of its common stock at $15.50 per share, resulting in gross proceeds of $300.0 million. Under the terms of the underwriting agreement, the Company granted the underwriters an option, exercisable for 30 days after February 16, 2018, to purchase up to an additional 2,903,225 shares of the Company's common stock, which was exercised with respect to 885,000 shares of the Company's common stock at a purchase price of $15.50 per share. The Company received net proceeds of $294.6 million from these offerings, after deducting underwriting discounts and commissions and offering expenses payable by the Company.

In April 2018, 453,214 warrants were exercised at $7.98 per share of common stock resulting in gross cash proceeds of $3.6 million.

On June 7, 2018, the Company's stockholders approved an amendment to the Company's Restated Certificate of Incorporation to increase the number of shares of common stock, par value $0.01 per share, that the Company is authorized to issue from 250,000,000 shares to 500,000,000 shares.

Note 8. Share based Compensation

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 units 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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Expected stock price volatility	75%	82%	80%	83%
Risk free interest rate	2.7%	1.8%	2.4%	2.0%
Expected life of options (years)	5.62	5.89	5.62	6.21
Expected annual dividend per share	$ —	$ —	$ —	$ —

Beginning in the third quarter of 2017, the average expected life was determined using our actual historical data versus a "simplified" method used in prior quarters. The "simplified" method of estimating the expected exercise term uses the mid-point between the vesting date and the end of the contractual term. In earlier quarters, we did not have sufficient reliable exercise data to justify a change from the use of the "simplified" method of estimating the expected exercise term of employee stock option grants. The impact from this change was not material.

A summary of the Company's stock options for the nine months ended September 30, 2018 were as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Options outstanding, December 31, 2017	15,181	$ 7.48		
Granted	2,077	$ 15.36		
Exercised	(1,282)	$ 6.62		
Forfeited	(200)	$ 9.75		
Expired	(7)	10.45		
Options outstanding, September 30, 2018	15,769	$ 8.56	7.0 years	$ 65.8

Vested and unvested expected to vest, September 30, 2018	15,003	$ 8.43	6.9 years	$ 63.9
Exercisable at September 30, 2018	9,406	$ 7.26	6.0 years	$ 47.4

As of September 30, 2018, the total unrecognized compensation cost related to non-vested stock options granted was $36.0 million and is expected to be recognized over a weighted average period of three 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 nine months ended September 30, 2018 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Non-vested units as of December 31, 2017	2,575	$ 5.85		
Granted	1,635	$ 16.57		
Vested	(517)	$ 5.91		
Forfeited	(58)	$ 10.23		
Non-vested units as of September 30, 2018	3,635	$ 10.49	2.7 years	$ 44.0

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 2007 Equity Incentive Plan, including named executive officers. Certain awards under the Performance-Based RSU Agreement were granted in January 2017 and 2018. The 2018 grants include 187,222 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% (374,444 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 on a straight-line basis over the vesting term. The estimated fair value per share of the MPRSUs was $25.44 and was calculated using a Monte Carlo simulation model. The awards also include 187,211 performance based awards that will vest over the next three years based on the Company achieving certain clinical milestones. During the nine months ended September 30, 2018, none of the clinical milestones for the performance based RSUs awarded in 2017 or 2018 were reached.

For the nine months ended September 30, 2018, 516,940 RSUs have vested and all non-vested units are expected to vest over their normal term. As of September 30, 2018, there was $25.6 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 three 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:

	Three Months Ended September 30,	Nine Months Ended September 30,

(in thousands)	2018	2017	2018	2017
Equity compensation expense recognized in:				
Research and development expense	$2,905	$2,390	$8,603	$7,456
Selling, general and administrative expense	4,149	3,110	12,270	9,611
Total equity compensation expense	$7,054	$5,500	$20,873	$17,067

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 September 30, 2018 are identified in the following table:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	$82,655	$82,655
Asset-backed securities	52,101	52,101
Corporate debt securities	227,399	227,399
Money market funds	3,335	3,335
	$365,490	$365,490

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$—	$28,100	$28,100
Deferred compensation plan liability	2,996	—	2,996
	$2,996	$28,100	$31,096

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, 2017 are identified in the following table:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	$79,803	$79,803
Asset-backed securities	30,287	30,287
Corporate debt securities	199,012	199,012
Money market funds	2,598	2,598
	$311,700	$311,700

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$—	$25,400	$25,400
Deferred compensation plan liability	2,258	—	2,258
	$2,258	$25,400	$27,658

The Company's Convertible Notes fall into the 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 fair value of the debt at September 30, 2018 was approximately $531.2 million.

The Company's Senior Secured Term Loan fall into the Level 2 category within the fair value level hierarchy and the fair value was determined using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals. The carrying value of the Senior Secured Term Loan approximates the fair value.

The Company did not have any Level 3 assets as of September 30, 2018 or December 31, 2017.

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 an active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available for sale debt securities 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 nine months ended September 30, 2018. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the nine months ended September 30, 2018.

Contingent Consideration Payable

The contingent consideration payable resulted from the acquisition of Callidus Biopharma, Inc. ("Callidus") in November 2013. 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.

The contingent consideration payable for 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.

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 September 30, 2018	Valuation Technique	Unobservable Input	Range
			Discount rate	10.0%
Clinical and regulatory milestones	$27.6 million	Probability weighted discounted cash flow	Probability of achievement of milestones	71.0%-100.0%
			Projected year of payments	2018-2022

Contingent consideration liabilities are remeasured to fair value each reporting period using 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. 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 and nine months ended September 30, 2018 and 2017, respectively:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2018	2017	2018	2017
Balance, beginning of the period	$26,800	$265,350	25,400	269,722
Payment of contingent consideration in cash	—	—	—	(10,000)
Changes in fair value during the period, included in statement of operations	1,300	(244,250)	2,700	(238,622)
Balance, end of the period	$28,100	$21,100	28,100	$21,100

Deferred Compensation Plan - Investment and Liability

The Deferred Compensation Plan (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 investment’s 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.

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:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except per share amounts)	2018	2017	2018	2017
Numerator:				
Net loss attributable to common stockholders	$(159,163)	$(111,666)	$(270,914)	$(214,794)
Denominator:				
Weighted average common shares outstanding — basic and diluted	189,162,841	160,796,841	184,606,790	148,963,864

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:

	As of September 30,	
(in thousands)	2018	2017
Options to purchase common stock	15,769	16,212
Convertible notes	40,850	40,850
Outstanding warrants, convertible to common stock	2,657	3,110
Unvested restricted stock units	3,635	2,690
Vested restricted stock units, unissued	103	50

Total number of potentially issuable shares	63,014	62,912

Note 11. Collaborations

In October 2018, the Company further expanded its gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for four additional indications, including Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder. This relationship will combine the Company's protein engineering and glycobiology expertise with Penn’s AAV gene transfer technologies to develop AAV gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability. In connection with the collaboration agreement, the Company made an upfront payment of $7 million in cash to Penn in October 2018 and agreed to certain milestone payments following the achievement of certain developmental and commercial milestone events by a licensed product in each indication up to an aggregate of $86.5 million per indication.

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

Overview

We are a global patient-centric biotechnology company engaged in the discovery, development and commercialization of a diverse set of novel treatments for patients living with rare metabolic diseases. With one medicine for Fabry disease achieving widespread global approval, a differentiated biologic for Pompe disease in the clinic and the recent addition of fourteen new gene therapy programs into our pipeline, including two clinical stage gene therapies for Batten disease, we have a leading portfolio of medicines for lysosomal storage disorders ("LSDs").

The cornerstone of our portfolio is migalastat HCl, (also referred to as "migalastat"), the first and only approved oral precision medicine for people living with Fabry disease who have amenable genetic variants. Migalastat is currently approved under the trade name Galafold® in the United States ("U.S."), European Union ("EU") and Japan, with additional approvals granted and applications pending in several geographies. During the third quarter of 2018, we initiated the commercial launch of Galafold® in the U.S. for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant.

The lead biologics program of our pipeline is Amicus Therapeutics GAA ("AT-GAA", also known as ATB200/AT2221), a novel, clinical-stage, potential best-in-class treatment paradigm for Pompe disease. Our Chaperone-Advanced Replacement Therapy ("CHART®") platform technology is leveraged to develop novel products for Pompe disease and potentially future other LSDs.

During the second half of 2018, we have expanded our portfolio to include fourteen new gene therapy programs. During the third quarter of 2018, we acquired worldwide development and commercial rights for ten gene therapy programs for neurologic LSDs developed at The Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital ("NCH") and The Ohio State University through the acquisition of Celenex, Inc. ("Celenex"), a private, clinical stage gene therapy company, for cash consideration of $100.0 million and additional consideration payable upon the achievement of certain development and approval milestones. The acquisition establishes Amicus as a leading company in neurologic LSDs. The lead programs in CLN6, CLN3, and CLN8 Batten disease are potential first-to-market curative therapies for these rare, devastating diseases.

In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania ("Penn") to pursue the research and development of novel gene therapies for four additional indications, including Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD") and one additional undisclosed rare metabolic disorder. This relationship will combine our protein engineering and glycobiology expertise with Penn's adeno associated virus ("AAV") gene transfer technologies to develop AAV gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.
We believe that our platform technologies and our product pipeline uniquely position us and drive our commitment to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

During the third quarter of 2018, we entered into a loan agreement with BioPharma Credit PLC, as the lender, for a $150.0 million non-dilutive senior secured term loan (the "Senior Secured Term Loan") with an interest rate equal to 3-month LIBOR plus 7.50% per annum, subject to a floor and ceiling on the rate, which matures in five years. We

received net proceeds of $146.6 million in September 2018, after deducting fees and estimated expenses payable by us. There are no warrants or any equity conversion features associated with the Senior Secured Term Loan. The proceeds from this financing will be used to support the cost of the Celenex acquisition, its related development costs and other general corporate purposes.

Our Strategy

Our strategy is to create, manufacture, test and deliver the highest quality medicines for people living with rare metabolic diseases through internally developed, acquired or in-licensed products and product candidates that have the potential to obsolete current treatments, provide significant benefits to patients, and be first- or best-in-class. In addition to our lead programs in Fabry and Pompe, we have begun to leverage our global capabilities to develop and expand our robust pipeline through our recent entry into genomic medicine. Since the beginning of the year, we made significant progress toward fulfilling our vision to build a leading global biotechnology company focused on rare metabolic diseases.

Highlights of our progress in 2018 include:

- Commercial success. In the nine months ended September 30, 2018, Galafold® revenue totaled approximately $58.6 million. Revenue has been generated primarily in the EU.
- Regulatory progress. We received approval for migalastat in the U.S. and Japan.
- Pompe clinical study. We have reported positive data from a Phase 1/2 clinical study to evaluate Pompe disease patients treated with our novel treatment paradigm AT-GAA.
- Pipeline Growth: With 14 new gene therapy programs for LSDs, we have established a leading portfolio of medicines for people living with rare metabolic disorders. We acquired worldwide development and commercial rights for ten gene therapy programs in rare, neurologic LSDs with lead programs in CLN6, CLN3, and CLN8 Batten disease. An additional four programs were added to the pipeline through the collaboration with Penn to pursue research and development of novel gene therapies for Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder.
- Manufacturing. We successfully scaled manufacture of Pompe biologic engineering batches to commercial scale (1,000L) with capacity plans to enable us to produce sufficient quantities to serve the entire Pompe population as quickly as possible after receipt of applicable regulatory approvals. Through our collaborations with NCH and Penn, we also gain access to their preclinical manufacturing capabilities, clinical supply and CMO relationships for those programs.
- Financial strength. Total cash, cash equivalents and marketable securities of $564.4 million at September 30, 2018 compared to $358.6 million at December 31, 2017. The current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe and gene therapy program operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact our future capital requirements.

Our Commercial Product and Product Candidates

Migalastat for Fabry Disease

Migalastat was granted accelerated approval by the U.S. Food and Drug Administration (“FDA”) in August 2018 under the brand name Galafold® for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene ("GLA") variant based on in vitro assay data. The FDA approved Galafold® for 348 amenable GLA variants. Migalastat was approved 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 variant. The approved EU label includes 367 Fabry-causing variants, which represent up to half of all patients with Fabry disease. Approvals have also been granted in Australia, Canada, Israel, Japan, South Korea, and Switzerland, with additional applications pending in other geographies.

We have been granted pricing and reimbursement in 22 countries. We plan to continue to launch Galafold® in additional countries during the remainder of 2018 and 2019.

As an orally administered monotherapy, migalastat is designed to bind to and stabilize an endogenous alpha-galactosidase A (“alpha-Gal A”) enzyme in those patients with genetic variants identified as amenable in a GLP cell-based amenability assay. Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic variants and, at this time, it is not intended for concomitant use with ERT.

Patients with Fabry disease have an inherited deficiency of the alpha-Gal A enzyme that would normally degrade the lipid substrate globotriaosylceramide in the lysosome. Genetic variants that cause changes in the amino acid sequence of alpha-Gal A result in an unstable enzyme that does not efficiently fold into its correct three-dimensional shape and cannot be trafficked properly in the cell, even if it has the potential for biological activity. Migalastat is an oral small

molecule pharmacological chaperone that is designed to bind to and stabilize a patient's own endogenous target protein. This is considered a precision medicine because migalastat targets only patients with GLA variants (mutations) amenable to migalastat.

Next-Generation Gene Therapy for Fabry Disease

We are committed to continued innovation for all people living with Fabry disease. For people living with Fabry disease who have non-amenable variants (mutations), which are not suitable for migalastat as a monotherapy, our strategy is to develop a Fabry gene therapy. In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for Fabry disease. For additional information, see above "—Overview."

Novel ERT for Pompe Disease

We are leveraging our biologics capabilities and CHART® platform to develop AT-GAA, a novel treatment paradigm for Pompe disease. AT-GAA consists of a uniquely engineered rhGAA enzyme, ATB200, with an optimized carbohydrate structure to enhance lysosomal 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, Inc. ("Callidus"). ATB200 is our first biologic to enter clinical development.

The pharmacological chaperone, AT2221 is not an active ingredient that contributes directly to GAA substrate reduction but instead acts to stabilize ATB200. The small molecule pharmacological chaperone AT2221 binds and stabilizes ATB200 to improve the uptake of active enzyme in key disease-relevant tissues, resulting in increased clearance of accumulated substrate, glycogen.

In preclinical studies, AT-GAA demonstrated greater tissue enzyme levels and further substrate reduction compared to the currently approved ERT for Pompe disease (alglucosidase alfa).

We have been engaged in collaborative discussions with U.S. and EU regulators regarding a registration-directed study for approval, manufacturing activities, and the best and fastest pathway forward for AT-GAA. Following feedback from the FDA and the scientific advice received from the European Medicines Agency ("EMA"), we plan to initiate a pivotal study in the second half of 2018. Both regulatory agencies indicated that the current clinical package is not sufficient to support accelerated approval or conditional approval. We continue to generate data to support further discussions on a potential pathway for accelerated approval with the FDA and conditional approval with the EMA authorities in 2019.

On October 5, 2018 we reported additional interim data from our clinical study ATB200-02 at the 23rd International Annual Congress of the World Muscle Society. Highlights included safety and tolerability data in all 20 patients (maximum of 28+ months of treatment) as well as PD data (muscle damage biomarker and disease substrate biomarker) for all 20 patients (15 ERT-switch patients and 5 ERT-naive patients). To date, adverse events have been generally mild and transient. AT-GAA has resulted in a low rate of infusion-associated reactions ("IARs") following 890+ infusions (seven events of IARs in five patients; < 1% of all 890+ infusions with an IAR). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data. Treatment with AT-GAA resulted in persistent and durable reductions in creatine kinase and urine hexose tetrasaccharide across all patient cohorts out to month 18.

As of the last interim analysis in October 2018, data on functional outcomes are available for 19 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations). Muscle function improved in 17 of 19 patients at month 12. Muscle function improved in 17 out of 18 patients with available data at month 18. Mean 6MWT improved in both ERT-naïve and ERT-switch patients with continued benefit observed out to month 18. All 5 ERT-naïve patients showed increases in 6MWT distance at all-time points out to month 18. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 63 meters at month 12 (n=5), and 49 meters at month 18 (n=5). 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6,12

and 18, respectively. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 42 meters at month 12 (n=10), and 52 meters at month 18 (n=9). Other motor function tests generally showed mean improvements consistent with 6MWT distance out to month 18 in both ambulatory cohorts. Three of the four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (which includes elbow and shoulder) from baseline to month 18, as measured by quantitative muscle testing and manual muscle testing. Pulmonary function improved in ERT-naïve patients and was generally stable in ERT-switch patients. In ERT-naïve patients, mean absolute change in forced vital capacity ("FVC") one of the main measures of pulmonary function in Pompe disease, was +4.2% at month 6 (n=5), +4.4% at month 12 (n=5), and +5.0% at month 18 (n=5). In ERT-switch patients mean absolute change in FVC was -1.3% at month 6 (n=9), -3.3% at month 12 (n=9), and -3.7% at month 18 (n=8). Overall, other pulmonary tests of maximal inspiratory pressure, a measure of inhalation, and maximal expiratory pressure, a measure of exhalation, were stable or increased in both ERT-naïve and ERT-switch patients.

Next-Generation Gene Therapy for Pompe Disease

We are committed to continued innovation for people living with Pompe disease. In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for, among other indications, Pompe disease. For additional information, see above "—Overview."

CDKL5 Deficiency Disorder

We are researching a potential first-in-class protein replacement therapy approach for CDD in addition to a gene therapy in preclinical studies. CDKL5 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 CDD cannot walk or care for themselves and may also suffer from scoliosis, visual impairment, sensory issues, and gastrointestinal complications.

In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for, among other indications, CDD. For additional information, see above "—Overview."

Strategic Alliances and Arrangements

In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for four additional indications, including Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder. For additional information, see above "—Overview."

We will continue to evaluate business development opportunities as appropriate that build stockholder value and provide us with access to the financial, technical, clinical, and commercial resources necessary to develop and market pharmacological chaperone therapeutics, ERTs, gene therapies and other technologies or products with a focus on rare metabolic diseases. We are exploring potential collaborations, alliances, and other business development opportunities on a regular basis. These opportunities may include the acquisition of preclinical-stage, clinical-stage or marketed products so long as such transactions are consistent with our strategic plan to develop and provide therapies to patients living with rare and orphan diseases.

Consolidated Results of Operations

Three Months Ended September 30, 2018 compared to September 30, 2017

The following table provides selected financial information for the Company:

	Three Months Ended September 30,		
(in thousands)	2018	2017	Change
Net product sales	$20,596	$10,874	$9,722
Cost of goods sold	4,310	1,790	2,520
Cost of goods sold as a percentage of net product sales	20.9 %	16.5 %	4.4 %
Operating expenses:			
Research and development	138,227	40,641	97,586
Selling, general and administrative	31,867	21,647	10,220
Changes in fair value of contingent consideration payable	1,300	(244,250)	245,550
Loss on impairment of asset	—	465,427	(465,427)
Depreciation	1,073	851	222
Other income (expense):			
Interest income	2,721	1,190	1,531
Interest expense	(4,715)	(4,351)	(364)
Change in fair value of derivatives	—	—	—
Other (expense) income	(1,039)	2,044	(3,083)
Income tax benefit	51	164,683	(164,632)
Net loss attributable to common stockholders	$(159,163)	$(111,666)	$(47,497)

Net Product Sales. Net product sales increased $9.7 million during the three months ended September 30, 2018 compared to the same period in the prior year. Galafold® was approved for sale in the EU in May 2016 and has been approved for pricing and reimbursement in 22 countries, including U.S. and Japan, as well as in select other European markets through reimbursed EAPs. The increase in revenue was related to the increase in the number of markets where we had obtained pricing and reimbursements and the corresponding increase in the number of patients being treated with Galafold®.

Cost of Goods Sold. Cost of goods sold includes manufacturing costs as well as royalties associated with sales of our product. Cost of goods sold as a percentage of net product sales was 20.9% during the three months ended September 30, 2018 compared to 16.5% during the same period in the prior year primarily due to the proportion of sales in countries subject to a higher royalty burden.

Research and Development Expense. The following table summarizes our principal product development programs for each product candidate in development, and the out-of-pocket, third party expenses incurred with respect to each product candidate:

(in thousands)	Three Months Ended September 30,	
Projects	2018	2017
Third party direct project expenses		
Migalastat (Fabry Disease)	$2,610	$3,265
AT-GAA (Pompe Disease)	12,312	10,456
SD-101 (EB-Epidermolysis Bullosa)	—	9,660
Pre-clinical programs	254	192
Total third party direct project expenses	15,176	23,573
Other project costs		
Personnel costs	15,584	11,410
Other costs	7,467	5,658
Total other project costs	23,051	17,068
Business development transactions	100,000	—
Total research and development costs	$138,227	$40,641

The increase in research and development costs was primarily due to $100 million in expense associated with the acquisition of ten gene therapy assets with the Celenex transaction. There were also increases in personnel and other costs with the advancement and enrollment of clinical studies and investments in manufacturing. The decrease in the EB program was due to the discontinuation of the program after the results of a Phase 3 study that did not meet the primary endpoint in September 2017.

Selling, General and Administrative Expense. Selling, general and administrative increased $10.2 million primarily due to the expanded geographic scope of the ongoing commercial launch of Galafold® and related operational costs of our global business.

Changes in Fair Value of Contingent Consideration Payable. The change in the fair value of the contingent consideration payable of $245.6 million resulted from a decrease in the Scioderm, Inc. (“Scioderm”) contingent consideration of $254.7 million, partially offset by an increase in the Callidus contingent consideration of $9.1 million. The fair value and change in fair value are impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods and rates. The decrease in Scioderm contingent consideration was due to the results announced in September 2017 that the study did not meet the primary or secondary endpoints, and, as a result, the contingent consideration is no longer payable.

Loss on Impairment of Assets. For the three months ended September 30, 2017, we recorded $465.4 million as impairment charges to assets, which primarily included $463.7 million in In-Process Research and Development (“IPR&D”). The impairment was assessed after the announcement of the results from the Phase 3 Scioderm ESSENCE study. There was no similar event in 2018.

Other Expense. The $3.1 million increase in other expense was primarily due to unrealized losses on foreign exchange transactions.

Income Tax Benefit. The income tax benefit is related to the provision for the three months ended September 30, 2018. We are subject to income taxes in the United States, although currently not a tax payer, and in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among

these different jurisdictions. The income tax benefit for the three months ended September 30, 2017 was $164.7 million and was primarily due to the reduction of the deferred tax liability related to Scioderm IPR&D as a result of announcement of the Phase 3 Scioderm ESSENCE Study in 2017.

Nine Months Ended September 30, 2018 compared to September 30, 2017

The following table provides selected financial information for the Company:

	Nine Months Ended September 30,		
(in thousands)	2018	2017	Change
Net product sales	$58,601	$22,201	36,400
Cost of goods sold	10,060	3,626	6,434
Cost of goods sold as a percentage of net product sales	17.2 %	16.3 %	0.9 %
Operating expenses:			
Research and development	213,685	103,502	110,183
Selling, general and administrative	88,435	60,090	28,345
Changes in fair value of contingent consideration payable	2,700	(238,622)	241,322
Loss on impairment of assets	—	465,427	(465,427)
Depreciation	3,015	2,486	529
Other income (expense):			
Interest income	7,371	2,702	4,669
Interest expense	(13,763)	(12,820)	(943)
Change in fair value of derivatives	(2,739)	163	(2,902)
Other (expense) income	(3,593)	4,891	(8,484)
Income tax benefit	1,104	164,578	(163,474)
Net loss attributable to common stockholders	$(270,914)	$(214,794)	$(56,120)

Net Product Sales. Net product sales increased $36.4 million during the nine months ended September 30, 2018 compared to the same period in the prior year. Galafold® was approved for sale in the EU in May 2016 and has been approved for pricing and reimbursement in 22 countries, including U.S. and Japan, as well as in select other European markets through reimbursed EAPs. The increase in revenue was related to the increase in the number of markets where we had obtained pricing and reimbursements and the corresponding increase in the number of patients being treated with Galafold®.

Cost of Goods Sold. Cost of goods sold includes manufacturing costs as well as royalties associated with sales of our product. Cost of goods sold as a percentage of net product sales increased to 17.2% during the nine months ended September 30, 2018 compared to 16.3% during the same period in the prior year primarily due to the proportion of sales in countries subject to a higher royalty burden.

Research and Development Expense. The following table summarizes our principal product development programs for each product candidate in development, and the out-of-pocket, third party expenses incurred with respect to each product candidate:

(in thousands)	Nine Months Ended September 30,	
Projects	2018	2017
Third party direct project expenses		
Migalastat (Fabry Disease)	$10,044	$8,644
AT-GAA (Pompe Disease)	37,364	29,594
SD-101 (EB-Epidermolysis Bullosa)	—	15,424
Pre-clinical programs	1,054	430
Total third party direct project expenses	48,462	54,092
Other project costs		
Personnel costs	44,501	34,162
Other costs	20,722	15,248

Total other project costs	65,223	49,410
Business development transactions	100,000	—
Total research and development costs	$213,685	$103,502

The increase in research and development costs was primarily due to $100 million in expenses associated with the acquisition of ten gene therapy assets with the Celenex transaction. There were also increases in personnel and other costs with the advancement and enrollment of clinical studies and investments in manufacturing. The decrease in the EB program was due to the discontinuation of the program after the results of a Phase 3 study that did not meet the primary endpoint in September 2017.

Selling, General and Administrative Expense. Selling, general and administrative increased $28.3 million primarily due to the expanded geographic scope of the ongoing commercial launch of Galafold®and related operational costs of our global business.

Changes in Fair Value of Contingent Consideration Payable. The change in the fair value of the contingent consideration payable of $241.3 million resulted from a decrease in the Scioderm contingent consideration of $250.0 million, partially offset by an increase in the Callidus contingent consideration of $8.7 million. The fair value and change in fair value are impacted by updates to the estimated probability of achievement, assumed timing of milestones and adjustments to the discount periods and rates. The decrease in Scioderm contingent consideration was due to the results announced in September 2017 that the study did not meet the primary or secondary endpoints, and, as a result, the contingent consideration is no longer payable.

Loss on Impairment of Assets. For the nine months ended September 30, 2017, we recorded $465.4 million as impairment charges to assets, which primarily included $463.7 million in IPR&D. The impairment was assessed after the announcement of the results from the Phase 3 ESSENCE Scioderm study. There was no similar event in 2018.

Interest Income. Interest income increased $4.7 million due to the overall higher average cash and investment balances as a result of our financing transactions.

Change in Fair Value of Derivatives. Subsequent to the underwritten public offering on February 15, 2018, we did not have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations at February 15, 2018 was determined to be $507.4 million and $13.6 million, respectively, of which the portion that was determined to not be able to be net share settled was recorded with a corresponding impact to additional-paid-in-capital. Following the approval by the Company's stockholders on June 7, 2018, to increase the authorized shares of common stock to 500,000,000, we now have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. As a result, the derivative liability and derivative asset were reclassified into additional-paid-in-capital. Subsequent changes to fair value of the derivatives were recorded through earnings on our consolidated statements of operations resulting in a change in fair value of derivatives for the nine months ended September 30, 2018 of $2.7 million.

Other Expense. The $8.5 million increase in other expense was primarily due to unrealized losses on foreign exchange transactions.

Income Tax Benefit. The income tax benefit recorded during the nine months ended September 30, 2018 related to a discrete tax item. We are subject to income taxes in the United States, although currently not a tax payer, and in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions. The income tax benefit for the nine months ended September 30, 2017 was $164.6 million and was primarily due to the reduction of the deferred tax liability related to Scioderm IPR&D as a result of announcement of the Phase 3 ESSENCE Study in 2017.

Liquidity and Capital Resources

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.

Source of Liquidity

During the third quarter of 2018, we entered into a loan agreement with BioPharma Credit PLC, as the lender, for a $150.0 million non-dilutive senior secured term loan (the "Senior Secured Term Loan") with an interest rate equal to 3-month LIBOR plus 7.50% per annum, subject to a floor and ceiling on the rate, which matures in five years. We received net proceeds of $146.6 million in September 2018, after deducting fees and estimated expenses payable by us.

During the first quarter of 2018, we issued, through an underwritten offering, 20,239,839 shares of our common stock resulting in net proceeds of $294.6 million after deducting underwriting discounts and commissions and offering expenses payable by us. We expect to use the net proceeds of the offering for investment in the U.S. and international commercial infrastructure for migalastat HCl, investment in manufacturing capabilities for ATB200, the continued clinical development of our product candidates, research and development expenditures, clinical and pre-clinical trial expenditures, commercialization expenditures and for other general corporate purposes.

Cash flow discussion

As of September 30, 2018, we had cash, cash equivalents and marketable securities of $564.4 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances. For more details on the cash, cash equivalents and marketable securities, refer to "—Note 4. Cash, Cash Equivalents, Marketable Securities and Restricted Cash," in our Notes to Consolidated Financial Statements.

Net Cash Used in Operating Activities

Net cash used in operations for the nine months ended September 30, 2018 was $239.3 million. The components of net cash used in operations included the net loss for the nine months ended September 30, 2018 of $270.9 million and the net increase in operating assets of $5.7 million. The change in operating assets was primarily due to increases in accounts receivable by $5.2 million and inventory of $2.0 million due to commercial sales of Galafold®, partially offset by a decrease in prepaid and other current assets of $2.6 million for spending to support commercial activities for Galafold® launch. The net cash used in operations was also impacted by an increase in accounts payable and accrued expenses of $3.7 million, mainly related to program expenses and support for the commercial launch of Galafold®, and a decrease in deferred reimbursement of $5.0 million due to payment of a milestone.

Net cash used in operations for the nine months ended September 30, 2017 was $140.7 million. The components of net cash used in operations included the net loss for the nine months ended September 30, 2017 of $214.8 million and the decrease in operating assets of $10.7 million and deferred reimbursement of $12.6 million. The decrease in operating assets was primarily due to increases in accounts receivable of $4.3 million, inventories by $3.4 million, and prepaid expenses and other current assets of $3.4 million due to an increase in commercial sales. The net cash used in operations was partially offset by an increase of $13.0 million in accounts payable and accrued expenses related to program expenses and support for the commercial launch of Galafold®.

Net Cash Used in Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2018 was $57.4 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 $441.0 million for the purchase of marketable securities, and $4.6 million for the acquisition of property and equipment, partially offset by $388.1 million for the sale and redemption of marketable securities.

Net cash used in investing activities for the nine months ended September 30, 2017 was $222.8 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 $450.4 million for the purchase of marketable securities, and $3.4 million for the acquisition of property and equipment, partially offset by $231.0 million for the sale and redemption of marketable

securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2018 was $450.4 million. Net cash provided by financing activities primarily reflects $294.6 million from the issuance of common stock, net of issuance costs, $146.6 million in proceeds from the Senior Secured Term Loan, net of issuance costs and estimated fees payable by the company, and $12.1 million from the exercise of stock options and warrants, partially offset by $2.7 million from the purchase of vested restricted stock units.

Net cash provided by financing activities for the nine months ended September 30, 2017 was $240.6 million. Net cash provided by financing activities primarily reflects $243.0 million from the issuance of common stock, net of issuance costs, $8.8 million from the exercise of stock options partially offset by $10.0 million in payment for contingent consideration, and $1.1 million from the purchase of vested of restricted stock units.

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 cost of manufacturing Pompe ERT;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates;
- 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 HCl;
- our ability to obtain market acceptance of migalastat HCl;
- 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 acquired 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 have generated revenue from product sales in 2018, 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 current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe and gene therapy program operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact our future capital requirements.

Financial Uncertainties Related to Potential Future Payments

Milestone Payments / Royalties

In September 2018, we expanded our pipeline by acquiring the rights and related intellectual property of ten gene therapy programs through our acquisition of Celenex. Celenex is a private, clinical stage gene therapy company whose lead programs are ten gene therapy programs including CLN6 and CLN3 which are in clinical stage, and several programs in pre-clinical stage. Pursuant to the terms of the agreement, we acquired Celenex for cash consideration of $100 million. We also agreed to pay up to an additional $15 million in connection with the achievement of certain development milestones, $262 million in connection with the achievement of certain regulatory approval milestones across multiple programs and up to $75 million in tiered sales milestone payments.

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Celenex has an exclusive license agreement with NCH). Under this license agreement, NCH is eligible to receive development and sales based milestones of up to $7.8 million from us for each product.

In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for four additional indications, including Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder. Under this collaboration agreement, Penn is eligible to receive certain milestone and royalty payments with respect to licensed products for each indication. Milestone payments are payable following the achievement of certain development and commercial milestone events in each indication, up to an aggregate of $86.5 million per indication. Royalty payments are based on net sales of licensed products on a licensed product-by-licensed product and country-by-country basis.

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 Mount Sinai School of Medicine (“MSSM”). This agreement expires upon expiration of the last of the licensed patent rights, which occurred in 2018 in the U.S. and will be in 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 LSDs 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.

In November 2013, we entered into the Revised Agreement (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 earlier agreement entered into between us and GSK in July 2012 (the "Original Collaboration Agreement"). 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 United States. 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.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, 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 the nine months ended September 30, 2018, under the license agreements we paid $7.1 million in royalties and $5.0 million in sales-based milestones.

Critical Accounting Policies and Significant Judgments

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 nine months ended September 30, 2018 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 for the fiscal year ended December 31, 2017, except as it relates to the adoption of ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which is described below.

Revenue Recognition

Our net product sales consist of sales of Galafold® for the treatment of Fabry disease primarily in the EU. We have recorded revenue on sales where Galafold® is available either on a commercial basis or through a reimbursed EAP. Orders for Galafold® are generally received from pharmacies and the ultimate payor is typically a government authority.

We recognize revenue when our performance obligation with our customers have been satisfied, which occurs at a point in time when the pharmacies or distributors obtain control of Galafold®. The transaction price is determined based on fixed consideration in our customer contracts and is recorded net of estimates for variable consideration, which are third party discounts and rebates. The identified variable consideration is recorded as a reduction of revenue at the time revenues from sales of Galafold® are recognized. We recognize revenue to the extent that it is probable that a significant revenue reversal will not occur in a future period. These estimates may differ from actual consideration amount received and we evaluate these estimates each reporting period to reflect known changes in factors.

We elected the portfolio approach practical expedient in applying ASC Topic 606 to our identified revenue streams. Contracts within each revenue stream have similar characteristics and we believe the results of this approach would not differ materially than if we applied ASC Topic 606 to each individual contract.

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 believe that a 1% (100 basis points) change in average interest rates would either increase or decrease the market value of our investment portfolio by $1.6 million as of September 30, 2018. 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. 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.

For information regarding our exposure to certain market risks, see Item 7A, Quantitative and Qualitative Disclosures About Market Risk, in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017. There have been no material changes in our financial instrument portfolio or market risk exposures since our fiscal year ended December 31, 2017.

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

None.

ITEM 1A. RISK FACTORS

The following risk factor should be considered in addition to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017.

It is difficult to predict the time and cost of development and subsequent regulatory approval of gene therapy product candidates.

The regulatory approval process for gene therapy products is also extensive, lengthy, expensive, and uncertain. In addition, regulatory requirements governing gene therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Tissue and Advanced Therapies within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the National Institute of Health, or NIH, also potentially are subject to review by the Regulatory Affairs Certification, or RAC; however, NIH announced in 2014 that the RAC will only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's institutional biosafety committee as well as its institutional review board would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our gene therapy product candidates. Similarly, the European Commission may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.
These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our gene therapy product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our gene therapy product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business would be materially harmed.

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

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

None.

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
2.1	Agreement and Plan of Merger, dated as of September 19, 2018, by and among Amicus Therapeutics, Inc.,Columbus Merger Sub Corp., Celenex, Inc. and Shareholder Representative Services LLC, solely in its capacity as the Shareholders' Representative (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K filed on September 25, 2018 (film no. 181084698)).
10.1	Loan Agreement, dated as of September 19, 2018, by and among Amicus Therapeutics, Inc., as Borrower, certain subsidiaries of the Borrower, as Guarantors, and Biopharma Credit PLC, as Lender (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed September 25, 2018 (film no. 181084701)).
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 nine months ended September 30, 2018, 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

SIGNATURES

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

AMICUS THERAPEUTICS, INC.

Date: November 5, 2018 By: /s/ John F. Crowley
John F. Crowley
Chairman and Chief Executive Officer
(Principal Executive Officer)

Date: November 5, 2018 By: /s/ William D. Baird III
William D. Baird III
Chief Financial Officer
(Principal Financial Officer)