

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2019

OR

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

For the transition period from to

Commission file number 001-33497

Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of
Incorporation or Organization)

71-0869350

(I.R.S. Employer
Identification Number)

1 Cedar Brook Drive, Cranbury, NJ
(Address of Principal Executive Offices)

08512
(Zip Code)

(609) 662-2000

(Registrant's Telephone Number, Including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---------------------|-------------------|---|
| Common Stock | FOLD | NASDAQ Global Market |

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

Indicate by check mark whether the registrant has submitted electronically 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 No

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

Non-accelerated filer

Accelerated filer

Smaller reporting company

Emerging growth company

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

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

The number of shares outstanding of the registrant's common stock, \$0.01 par value per share, as of April 29, 2019 was 230,450,221 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended March 31, 2019

| | <u>Page</u> |
|--|-------------|
| <u>PART I. FINANCIAL INFORMATION</u> | <u>3</u> |
| Item 1. <u>Consolidated Financial Statements and Notes (unaudited)</u> | <u>3</u> |
| <u>Consolidated Balance Sheets as of March 31, 2019 and December 31, 2018</u> | <u>3</u> |
| - | |
| <u>Consolidated Statements of Operations for the Three Months Ended March 31, 2019 and 2018</u> | <u>4</u> |
| - | |
| <u>Consolidated Statements of Comprehensive Loss for the Three Months Ended March 31, 2019 and 2018</u> | <u>5</u> |
| <u>Consolidated Statements of Changes in Stockholders' Equity for the Three Months Ended March 31, 2019 and 2018</u> | |
| - | |
| <u>Consolidated Statements of Cash Flows for the Three Months Ended March 31, 2019 and 2018</u> | <u>7</u> |
| <u>Notes to Consolidated Financial Statements</u> | <u>8</u> |
| Item 2. <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u> | <u>23</u> |
| Item 3. <u>Quantitative and Qualitative Disclosures about Market Risk</u> | <u>33</u> |
| Item 4. <u>Controls and Procedures</u> | <u>33</u> |
| <u>PART II. OTHER INFORMATION</u> | <u>34</u> |
| Item 1. <u>Legal Proceedings</u> | <u>34</u> |
| Item 1A. <u>Risk Factors</u> | <u>34</u> |
| Item 2. <u>Unregistered Sales of Equity Securities and Use of Proceeds</u> | <u>34</u> |
| Item 3. <u>Defaults Upon Senior Securities</u> | <u>34</u> |
| Item 4. <u>Mine Safety Disclosures</u> | <u>34</u> |
| Item 5. <u>Other Information</u> | <u>34</u> |
| Item 6. <u>Exhibits</u> | <u>35</u> |
| <u>SIGNATURES</u> | <u>36</u> |

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. FABRAZYME, MYOZYME, LUMIZYME, and REPLAGAL are the property of their respective owners.

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," "likely," "may," "outlook," "plan," "potential," "predict," "project," "seek," "should," "will," "would," the negatives or plurals thereof and other words 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") and gene therapies;
- 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 and gene therapies for the treatment of rare genetic metabolic diseases;
- 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 Galafold® ("migalastat HCl");
- our ability to manufacture or supply sufficient clinical or commercial products;
- our ability to obtain reimbursement for Galafold®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold®;
- our ability to obtain market acceptance of Galafold®;
- 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;
- our ability to adjust to changes in European and United Kingdom markets as the United Kingdom leaves the European Union;
- 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, 2018, 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, 2018 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)

| | March 31, 2019 | December 31, 2018 |
|--|-------------------|----------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 96,349 | \$ 79,749 |
| Investments in marketable securities | 341,978 | 424,403 |
| Accounts receivable | 22,960 | 21,962 |
| Inventories | 8,167 | 8,390 |
| Prepaid expenses and other current assets | 13,592 | 16,592 |
| Total current assets | 483,046 | 551,096 |
| Operating and finance lease right-of-use assets, less accumulated amortization of \$2,111 and \$0 at March 31, 2019 and December 31, 2018, respectively | 36,308 | — |
| Property and equipment, less accumulated depreciation of \$15,794 and \$15,671 at March 31, 2019 and December 31, 2018, respectively | 13,286 | 11,375 |
| In-process research & development | 23,000 | 23,000 |
| Goodwill | 197,797 | 197,797 |
| Other non-current assets | 11,265 | 6,683 |
| Total Assets | \$ 764,702 | \$ 789,951 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable, accrued expenses, and other current liabilities | \$ 60,596 | \$ 80,625 |
| Deferred reimbursements | 2,750 | 5,500 |
| Operating and finance lease liabilities | 2,523 | — |
| Total current liabilities | 65,869 | 86,125 |
| Deferred reimbursements | 11,406 | 10,156 |
| Convertible notes | 22,052 | 175,006 |
| Senior secured term loan | 146,766 | 146,734 |
| Contingent consideration payable | 20,767 | 19,700 |
| Deferred income taxes | 6,465 | 6,465 |
| Operating and finance lease liabilities | 36,100 | — |
| Other non-current liabilities | 3,609 | 2,853 |
| Total liabilities | 313,034 | 447,039 |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Common stock, \$0.01 par value, 500,000,000 shares authorized, 230,180,714 and 189,383,924 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively | 2,347 | 1,942 |
| Additional paid-in capital | 1,970,607 | 1,740,061 |
| Accumulated other comprehensive loss: | | |
| Foreign currency translation adjustment | (1,309) | 495 |
| Unrealized gain (loss) on available-for-sale securities | 157 | (427) |
| Warrants | 12,387 | 13,063 |
| Accumulated deficit | (1,532,521) | (1,412,222) |
| Total stockholders' equity | 451,668 | 342,912 |
| Total Liabilities and Stockholders' Equity | \$ 764,702 | \$ 789,951 |

See accompanying notes to consolidated financial statements

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

| | Three Months Ended March 31, | |
|---|------------------------------|--------------------|
| | 2019 | 2018 |
| Revenue: | | |
| Net product sales | \$ 34,046 | \$ 16,696 |
| Cost of goods sold | 4,055 | 2,615 |
| Gross profit | 29,991 | 14,081 |
| Operating expenses: | | |
| Research and development | 64,593 | 40,798 |
| Selling, general and administrative | 44,303 | 27,396 |
| Changes in fair value of contingent consideration payable | 1,383 | 1,100 |
| Depreciation and amortization | 991 | 969 |
| Total operating expenses | 111,270 | 70,263 |
| Loss from operations | (81,279) | (56,182) |
| Other income (expense): | | |
| Interest income | 2,639 | 1,737 |
| Interest expense | (6,454) | (4,488) |
| Loss on exchange of convertible notes | (36,123) | — |
| Change in fair value of derivatives | — | 4,861 |
| Other income | 1,086 | 2,764 |
| Loss before income tax | (120,131) | (51,308) |
| Income tax (expense) benefit | (168) | 1,392 |
| Net loss attributable to common stockholders | \$ (120,299) | \$ (49,916) |
| Net loss attributable to common stockholders per common share — basic and diluted | \$ (0.56) | \$ (0.28) |
| Weighted-average common shares outstanding — basic and diluted | 213,519,287 | 175,977,700 |

See accompanying notes to consolidated financial statements

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

| | Three Months Ended March 31, | |
|---|-------------------------------------|-------------|
| | 2019 | 2018 |
| Net loss | \$ (120,299) | \$ (49,916) |
| Other comprehensive (loss) gain: | | |
| Foreign currency translation adjustment gain (loss), net of tax impact of \$0 and \$0, respectively | (1,804) | (1,805) |
| Unrealized gain (loss) on available-for-sale securities | 584 | (441) |
| Other comprehensive income (loss) | \$ (1,220) | \$ (2,246) |
| Comprehensive loss | \$ (121,519) | \$ (52,162) |

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Changes in Stockholders' Equity
(Unaudited)
(in thousands, except share amounts)

Three Months Ended March 31, 2019

| | Common Stock | | Additional Paid-In Capital | Warrants | Other Comprehensive Gain (Loss) | Accumulated Deficit | Total Stockholders' Equity |
|--|--------------|----------|----------------------------------|-----------|---------------------------------------|------------------------|----------------------------------|
| | Shares | Amount | | | | | |
| Balance at December 31, 2018 | 189,383,924 | \$ 1,942 | \$ 1,740,061 | \$ 13,063 | \$ 68 | \$ (1,412,222) | \$ 342,912 |
| Stock issued from exercise of stock options, net | 578,451 | 6 | 3,947 | — | — | — | 3,953 |
| Restricted stock tax vesting | 301,058 | — | (1,940) | — | — | — | (1,940) |
| Stock issued for contingent consideration | 771,804 | 8 | 9,308 | — | — | — | 9,316 |
| Stock-based compensation | — | — | 12,744 | — | — | — | 12,744 |
| Warrants exercised | 101,787 | 1 | 1,487 | (676) | — | — | 812 |
| Equity component of the convertible notes | 39,043,690 | 390 | 190,368 | — | — | — | 190,758 |
| Termination of capped call confirmations | — | — | 14,632 | — | — | — | 14,632 |
| Unrealized holding gain on available-for-sale securities | — | — | — | — | 584 | — | 584 |
| Foreign currency translation adjustment | — | — | — | — | (1,804) | — | (1,804) |
| Net loss | — | — | — | — | — | (120,299) | (120,299) |
| Balance at March 31, 2019 | 230,180,714 | \$ 2,347 | \$ 1,970,607 | \$ 12,387 | \$ (1,152) | \$ (1,532,521) | \$ 451,668 |

Three Months Ended March 31, 2018

| | Common Stock | | Additional Paid-In Capital | Warrants | Other Comprehensive Gain (Loss) | Accumulated Deficit | Total Stockholders' Equity |
|---|--------------|----------|----------------------------------|-----------|---------------------------------------|------------------------|----------------------------------|
| | Shares | Amount | | | | | |
| Balance at December 31, 2017 | 166,989,790 | \$ 1,721 | \$ 1,400,758 | \$ 16,076 | \$ (2,095) | \$ (1,063,610) | \$ 352,850 |
| Stock issued from exercise of options, net | 560,721 | 6 | 3,972 | — | — | — | 3,978 |
| Stock issued from equity financing | 20,239,839 | 202 | 294,382 | — | — | — | 294,584 |
| Restricted stock tax vesting | 181,868 | — | (1,912) | — | — | — | (1,912) |
| Stock issued for contingent consideration | — | — | — | — | — | — | — |
| Stock-based compensation | — | — | 7,478 | — | — | — | 7,478 |
| Reclassification upon ASU 2018-02 adoption | — | — | — | — | (383) | 383 | — |
| Change in fair value of derivatives | — | — | (83,199) | — | — | — | (83,199) |
| Unrealized holding gain (loss) on available-for-sale securities | — | — | — | — | (441) | — | (441) |
| Foreign currency translation adjustment | — | — | — | — | (1,805) | — | (1,805) |
| Change in fair value of derivatives | — | — | — | — | — | — | — |
| Net loss | — | — | — | — | — | (49,916) | (49,916) |
| Balance at March 31, 2018 | 187,972,218 | \$ 1,929 | \$ 1,621,479 | \$ 16,076 | \$ (4,724) | \$ (1,113,143) | \$ 521,617 |

See accompanying notes to consolidated financial statements

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

| | Three Months Ended March 31, | |
|---|-------------------------------------|-------------------|
| | 2019 | 2018 |
| Operating activities | | |
| Net loss | \$ (120,299) | \$ (49,916) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Amortization of debt discount and deferred financing | 1,649 | 2,601 |
| Depreciation and amortization | 991 | 969 |
| Stock-based compensation | 12,744 | 7,478 |
| Loss on exchange of convertible debt | 36,123 | — |
| Change in fair value of derivatives | — | (4,861) |
| Non-cash changes in the fair value of contingent consideration payable | 1,383 | 1,100 |
| Foreign currency remeasurement (gain) loss | 325 | (2,957) |
| Changes in operating assets and liabilities: | | |
| Accounts receivable | (1,001) | (1,047) |
| Inventories | 345 | (3,253) |
| Prepaid expenses and other current assets | 3,059 | 10,875 |
| Accounts payable and accrued expenses | (8,858) | (10,347) |
| Other non-current assets and liabilities | (3,507) | (163) |
| Deferred reimbursements | (1,500) | — |
| Net cash used in operating activities | \$ (78,546) | \$ (49,521) |
| Investing activities | | |
| Sale and redemption of marketable securities | 135,187 | 121,226 |
| Purchases of marketable securities | (52,178) | (303,010) |
| Capital expenditures | (2,944) | (819) |
| Net cash used in investing activities | \$ 80,065 | \$ (182,603) |
| Financing activities | | |
| Proceeds from issuance of common stock, net of issuance costs | — | 294,584 |
| Payment of finance leases | (75) | (71) |
| Purchase of vested restricted stock units | (1,938) | (1,912) |
| Proceeds from termination of capped call confirmations | 14,632 | — |
| Proceeds from exercise of stock options | 3,953 | 3,978 |
| Proceeds of exercise of warrants | 812 | — |
| Net cash provided by financing activities | \$ 17,384 | \$ 296,579 |
| Effect of exchange rate changes on cash, cash equivalents and restricted cash | \$ (2,350) | \$ 860 |
| Net decrease in cash, cash equivalents and restricted cash | 16,553 | 65,315 |
| Cash, cash equivalents and restricted cash at beginning of period | \$ 82,375 | \$ 51,237 |
| Cash, cash equivalents and restricted cash at end of period | \$ 98,928 | \$ 116,552 |
| Supplemental disclosures of cash flow information | | |
| Cash paid during the period for interest | \$ 4,846 | \$ 12 |
| Capital expenditures, unpaid | \$ — | \$ 142 |
| Payment of contingent consideration in shares | \$ 9,316 | \$ — |

See accompanying notes to consolidated financial statements

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

Note 1. Business

Amicus Therapeutics, Inc. (the "Company") is a global patient-dedicated 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 therapies for lysosomal storage disorders ("LSDs").

The cornerstone of the Company's portfolio is Galafold® (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. In February 2019, the U.S. Food and Drug Administration ("FDA") granted Breakthrough Therapy Designation to AT-GAA for the treatment of late onset Pompe disease. The Company's Chaperone-Advanced Replacement Therapy ("CHART ®") platform technology is leveraged to develop novel products for Pompe disease and potentially other LSDs in the future.

With 14 new gene therapy programs, the Company has established a leading portfolio of medicines for people living with rare metabolic disorders. Through a license with NCH and collaboration with Penn, the Company's pipeline includes gene therapy programs in rare, neurologic LSDs with lead programs in CLN6, CLN3, and CLN8 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD") and one additional undisclosed rare metabolic disorder.

The Company believes that its platform technologies and 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 first quarter of 2019, the Company entered into separate, privately negotiated exchange agreements with a limited number of holders (the "Holders") of the Convertible Notes. Under the terms of the exchange agreements, the Holders agreed to exchange an aggregate principal amount of approximately \$219.3 million of Convertible Notes held by them in exchange for an aggregate of approximately 39.0 million shares of the our common stock, par value \$0.01 per share.

The Company had an accumulated deficit of approximately \$1.5 billion as of March 31, 2019 and anticipates incurring losses through the fiscal year ending December 31, 2019 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, 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 mid-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, 2018. 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, 2018.

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, Marketable Securities and Restricted Cash

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 March 31, 2019 have arisen from product sales primarily in the EU and U.S. 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. During the three months ended March 31, 2019, the Company recorded an allowance for doubtful accounts of \$0.3 million.

Revenue Recognition

The Company's net product sales consist of sales of Galafold® for the treatment of Fabry disease. 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 distributors and pharmacies with the ultimate payor often 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 following table summarizes the Company's net product sales from Galafold® disaggregated by geographic area:

| (in thousands) | Three Months Ended March 31, | |
|-------------------------|-------------------------------------|-------------|
| | 2019 | 2018 |
| U.S. | \$ 9,068 | \$ — |
| Ex-U.S. | 24,978 | 16,696 |
| Total net product sales | \$ 34,046 | \$ 16,696 |

Inventories and Cost of Goods Sold

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

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

Leases

In the ordinary course of business, the Company enters into lease agreements for office space as well as leases for certain property and equipment. The leases have varying terms and expirations and have provisions to extend or renew the lease agreement, among other terms and conditions, as negotiated. The Company determines if an arrangement is a lease at inception. Operating and finance leases are included in right-of-use ("ROU") assets and lease liabilities on the Company's consolidated balance sheets.

ROU assets represent the Company's right to control the use of an explicitly or implicitly identified fixed asset for a period of time and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term.

Lease payments included in the measurement of the lease liability are comprised of fixed payments. Variable lease payments are excluded from the ROU assets and lease liabilities and are recognized in the period in which the obligation for those payments are incurred. Variable lease payments are presented in the Company's consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all underlying asset categories.

A lessee is required to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments.

The lease term for all the Company's leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Leases with an initial term of 12 months or less are not recorded on the balance sheet. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. For additional information, see "—Note 9. Leases" and "—Note 2. Summary of Significant Accounting Policies, Recent Accounting Developments - Guidance Adopted in 2019."

Recent Accounting Developments - Guidance Adopted in 2019

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. Effective January 1, 2019 the Company adopted ASU 2016-02, along with the amendments issued in 2017 and 2018, and elected the transition method in ASU 2018-11. The Company elected the package of transition provisions available for expired or existing contracts, which allowed the Company to carry forward its historical assessments of (i) whether contracts are or contain leases, (ii) lease classification and (iii) initial direct costs. In addition, the Company applied the short-term lease recognition exemption for leases with terms at inception not greater than 12 months and will apply the practical expedient not to separate lease and non-lease components for new and modified leases commencing after adoption. The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. Upon adoption, the Company recorded a lease liabilities with a corresponding right-of-use assets of \$17.6 million. The adoption did not have a material impact on the consolidated results of operations and cash flows for the three-months ended March 31, 2019.

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 adopted the guidance in this Form 10-Q for the period ended March 31, 2019.

Recent Accounting Developments - Guidance Not Yet Adopted

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 2017-08 — 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 2016-13 — In June 2016, the FASB issued ASU 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). ASU 2016-13 requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected and amends guidance on the impairment of financial instruments. ASU 2016-13 is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. The Company expects to adopt this guidance when effective and is currently assessing the impact that this standard will have on its consolidated financial statements.

Note 3. Cash, Cash Equivalents, Marketable Securities and Restricted Cash

As of March 31, 2019, the Company held \$96.3 million in cash and cash equivalents and \$342.0 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 March 31, 2019 | | | | |
|--|-------------------|-----------------------------|-----------------------------|-------------------|
| (in thousands) | Cost | Gross unrealized Gain | Gross unrealized Loss | Fair Value |
| Cash and cash equivalents | \$ 96,349 | \$ — | \$ — | \$ 96,349 |
| Corporate debt securities, current portion | 138,194 | 96 | (6) | 138,284 |
| Commercial paper | 134,948 | 52 | (7) | 134,993 |
| Asset-backed securities | 68,278 | 30 | (8) | 68,300 |
| Money market | 350 | — | — | 350 |
| Certificates of deposit | 51 | — | — | 51 |
| | <u>\$ 438,170</u> | <u>\$ 178</u> | <u>\$ (21)</u> | <u>\$ 438,327</u> |
| Included in cash and cash equivalents | \$ 96,349 | \$ — | \$ — | \$ 96,349 |
| Included in marketable securities, current and non-current | 341,821 | 178 | (21) | 341,978 |
| Total cash, cash equivalents and marketable securities | <u>\$ 438,170</u> | <u>\$ 178</u> | <u>\$ (21)</u> | <u>\$ 438,327</u> |

| As of December 31, 2018 | | | | |
|--|-------------------|-----------------------------|-----------------------------|-------------------|
| (in thousands) | Cost | Gross unrealized Gain | Gross unrealized Loss | Fair Value |
| Cash and cash equivalents | \$ 79,749 | \$ — | \$ — | \$ 79,749 |
| Corporate debt securities, current portion | 240,969 | 7 | (250) | 240,726 |
| Commercial paper | 115,245 | — | (104) | 115,141 |
| Asset-backed securities | 68,215 | 4 | (84) | 68,135 |
| Money market | 350 | — | — | 350 |
| Certificates of deposit | 51 | — | — | 51 |
| | <u>\$ 504,579</u> | <u>\$ 11</u> | <u>\$ (438)</u> | <u>\$ 504,152</u> |
| Included in cash and cash equivalents | \$ 79,749 | \$ — | \$ — | \$ 79,749 |
| Included in marketable securities | 424,830 | 11 | (438) | 424,403 |
| Total cash, cash equivalents and marketable securities | <u>\$ 504,579</u> | <u>\$ 11</u> | <u>\$ (438)</u> | <u>\$ 504,152</u> |

For the three months ended March 31, 2019 there were no realized gains. For the fiscal year ended December 31, 2018, there were nominal realized gains. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available-for-sale debt securities as of March 31, 2019 and December 31, 2018 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 \$100.4 million and \$403.1 million as of March 31, 2019 and December 31, 2018, respectively.

The following table provides a reconciliation of cash and cash equivalents and restricted cash reported within the consolidated balance sheets that sum to the total of the same such amounts shown in the Company's consolidated statements of cash flows.

| (in thousands) | March 31, 2019 | December 31, 2018 | March 31, 2018 | December 31, 2017 |
|--|------------------|-------------------|-------------------|-------------------|
| Cash and cash equivalents | \$ 96,349 | \$ 79,749 | \$ 114,322 | \$ 49,060 |
| Restricted cash | 2,579 | 2,626 | 2,230 | 2,177 |
| Cash and cash equivalents and restricted cash shown in the statement of cash flows | <u>\$ 98,928</u> | <u>\$ 82,375</u> | <u>\$ 116,552</u> | <u>\$ 51,237</u> |

Note 4. 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) | March 31, 2019 | December 31, 2018 |
|--------------------------|-----------------|-------------------|
| Raw materials | \$ 1,321 | \$ 1,291 |
| Work-in-process | 3,041 | 3,485 |
| Finished goods | 3,805 | 3,614 |
| Total inventories | <u>\$ 8,167</u> | <u>\$ 8,390</u> |

The Company recorded a reserve for inventory of \$0.2 million as of March 31, 2019 and December 31, 2018.

Note 5. 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 5 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.

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 through earnings on the Company's consolidated statements of operations resulting in a change in fair value of derivatives for the three months ended March 31, 2018 of \$4.9 million. As of March 31, 2018, the Company recorded the fair value of the derivative liability of \$80.6 million as a current liability and the fair value of the Capped Call Confirmation of \$2.2 million as a current asset within Other Current Assets on the consolidated balance sheets.

During the first quarter of 2019, the Company entered into separate, privately negotiated exchange agreements with a limited number of holders (the "Holders") of the Convertible Notes. Under the terms of the exchange agreements (the "Exchange Agreements"), the Holders agreed to exchange an aggregate principal amount of approximately \$219.3 million of Convertible Notes held by them in exchange for an aggregate of approximately 39.0 million shares of the our common stock, par value \$0.01 per share. In addition, pursuant to the Exchange Agreements, the Company made aggregate cash payments of approximately \$1.0 million to the Holders to satisfy accrued and unpaid interest to the closing date of the transaction, along with cash in lieu of fractional shares. As a result of this exchange, the Company recognized a loss on exchange of debt of \$36.1 million in the consolidated statement of operations, and \$190.4 million in additional paid-in-capital and common stock of \$0.4 million in the consolidated balance sheets for the three months ended March 31, 2019.

During the first quarter of 2019, the Company also terminated the proportion of the Capped Call Confirmations related to the exchange of the Convertible Notes during the first quarter of 2019 for proceeds of approximately \$14.6 million.

The Convertible Notes and Senior Secured Term Loan consist of the following:

| Liability component (in thousands) | March 31, 2019 | December 31, 2018 |
|------------------------------------|-------------------|-------------------|
| Principal | 180,702 | \$ 400,000 |
| Less: debt discount (1) | (11,076) | (74,145) |
| Less: deferred financing (1) | (808) | (4,115) |
| Net carrying value of the debt | <u>\$ 168,818</u> | <u>\$ 321,740</u> |

(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 months ended March 31, 2019 and 2018, respectively:

| Interest component (in thousands) | Three Months Ended March 31, | |
|------------------------------------|------------------------------|-----------------|
| | 2019 | 2018 |
| Contractual interest expense | 4,813 | \$ 1,887 |
| Amortization of debt discount | 1,559 | 2,472 |
| Amortization of deferred financing | 83 | 129 |
| Total | <u>\$ 6,455</u> | <u>\$ 4,488</u> |

Note 6. Stockholders' Equity

During the three months ended March 31, 2019, 101,787 warrants were exercised at \$7.98 per share of common stock resulting in gross cash proceeds of \$0.8 million.

As discussed in "— Note 5. Debt" during the first quarter of 2019, the Company entered into separate, privately negotiated exchange agreements with a limited number of holders (the "Holders") of the Convertible Notes. Under the terms of the exchange agreements (the "Exchange Agreements"), the Holders agreed to exchange an aggregate principal amount of approximately \$219.3 million of Convertible Notes held by them in exchange for an aggregate of approximately 39.0 million shares of the our common stock, par value \$0.01 per share.

As further discussed in "— Note 8. Assets and Liabilities Measured at Fair Value," the Company reached a clinical milestone, which was the dosing of the first patient in a Phase 3 study, related to the contingent consideration from the acquisition of Callidus. The milestone for this event was \$9.0 million which was paid in Company stock during the three months ended March 31, 2019, resulting in \$9.3 million impact on stockholder's equity.

Note 7. 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, directors, advisors and consultants at a price to be determined by the Company's Board of Directors. The Plan is intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company's business. The 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. The Board of Directors, or its committee, is responsible for determining the individuals to be granted options, the number of options each individual will receive, the option price per share, and the exercise period of each option.

Stock Option Grants

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

| | Three Months Ended March 31, | |
|------------------------------------|------------------------------|-------|
| | 2019 | 2018 |
| Expected stock price volatility | 74.2% | 81.2% |
| Risk free interest rate | 2.5% | 2.3% |
| Expected life of options (years) | 5.68 | 5.67 |
| Expected annual dividend per share | \$ — | \$ — |

The average expected life is determined using our actual historical data.

A summary of the Company's stock options for the three months ended March 31, 2019 were as follows:

| | Number of Shares (in thousands) | Weighted Average Exercise Price | Weighted Average Remaining Contractual Life | Aggregate Intrinsic Value (in millions) |
|--|------------------------------------|---------------------------------|---|--|
| Options outstanding, December 31, 2018 | 15,810 | \$ 8.63 | | |
| Granted | 3,315 | \$ 10.15 | | |
| Exercised | (578) | \$ 6.83 | | |
| Forfeited | (195) | \$ 10.33 | | |
| Expired | (21) | \$ 10.77 | | |
| Options outstanding, March 31, 2019 | 18,331 | \$ 8.94 | 6.9 years | \$ 90.2 |
| Vested and unvested expected to vest, March 31, 2019 | 17,325 | \$ 8.84 | 6.8 years | \$ 87.1 |
| Exercisable at March 31, 2019 | 10,538 | \$ 7.84 | 5.5 years | \$ 62.8 |

As of March 31, 2019, the total unrecognized compensation cost related to non-vested stock options granted was \$44.1 million and is expected to be recognized over a weighted average period of three years.

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

RSUs awarded under the Plan are generally subject to graded vesting and are contingent on an employee's continued service. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse. A summary of non-vested RSU activity under the Plan for the three months ended March 31, 2019 is as follows:

| | Number of Shares (in thousands) | Weighted Average Grant Date Fair Value | Weighted Average Remaining Years | Aggregate Intrinsic Value (in millions) |
|--|------------------------------------|--|----------------------------------|--|
| Non-vested units as of December 31, 2018 | 3,712 | \$ 10.59 | | |
| Granted | 3,031 | \$ 10.91 | | |
| Vested | (600) | \$ 9.07 | | |
| Forfeited | (133) | \$ 12.73 | | |
| Non-vested units as of March 31, 2019 | 6,010 | \$ 10.86 | 3.0 years | \$ 81.7 |

For the three months ended March 31, 2019, 599,734 RSUs have vested and all non-vested units are expected to vest over their normal term. As of March 31, 2019, there was \$48.2 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:

| (in thousands) | Three Months Ended March 31, | |
|---|------------------------------|-----------------|
| | 2019 | 2018 |
| Equity compensation expense recognized in: | | |
| Research and development expense | \$ 5,032 | \$ 3,057 |
| Selling, general and administrative expense | 7,712 | 4,421 |
| Total equity compensation expense | <u>\$ 12,744</u> | <u>\$ 7,478</u> |

Note 8. Assets and Liabilities Measured at Fair Value

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

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

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

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

A summary of the fair value of the Company's recurring assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2019 are identified in the following table:

| (in thousands) | Level 2 | Total | |
|--------------------------------------|-------------------|-------------------|------------------|
| Assets: | | | |
| Commercial paper | \$ 134,993 | \$ 134,993 | |
| Asset-backed securities | 68,300 | 68,300 | |
| Corporate debt securities | 138,284 | 138,284 | |
| Money market funds | 3,963 | 3,963 | |
| | <u>\$ 345,540</u> | <u>\$ 345,540</u> | |
| | | | |
| | Level 2 | Level 3 | Total |
| Liabilities: | | | |
| Contingent consideration payable | \$ — | \$ 20,767 | \$ 20,767 |
| Deferred compensation plan liability | 3,613 | — | 3,613 |
| | <u>\$ 3,613</u> | <u>\$ 20,767</u> | <u>\$ 24,380</u> |

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, 2018 are identified in the following table:

| (in thousands) | Level 2 | | Total |
|--------------------------------------|---------|---------|------------|
| Assets: | | | |
| Commercial paper | \$ | 115,141 | \$ 115,141 |
| Asset-backed securities | | 68,135 | 68,135 |
| Corporate debt securities | | 240,726 | 240,726 |
| Money market funds | | 3,082 | 3,082 |
| | \$ | 427,084 | \$ 427,084 |
| | | | |
| | | Level 2 | Level 3 |
| | | | Total |
| Liabilities: | | | |
| Contingent consideration payable | \$ | — | \$ 19,700 |
| Deferred compensation plan liability | | 2,732 | — |
| | \$ | 2,732 | \$ 19,700 |
| | | | \$ 22,432 |

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 March 31, 2019 was approximately \$71.6 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 March 31, 2019 or December 31, 2018.

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 three months ended March 31, 2019. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the three months ended March 31, 2019.

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 March 31, 2019 (in thousands) | Valuation Technique | Unobservable Input | Range |
|------------------------------------|---|---|--|-----------|
| | | | Discount rate | 10.3% |
| Clinical and regulatory milestones | \$ 20,538 | Probability weighted discounted cash flow | Probability of achievement of milestones | 75%-78% |
| | | | Projected year of payments | 2021-2022 |

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 Company reached a clinical milestone, which was the dosing of the first patient in a Phase 3 study, related to the contingent consideration from the acquisition of Callidus. The milestone for this event was \$9.0 million which was paid in Company stock during the three months ended March 31, 2019, resulting in \$9.3 million impact on stockholder's equity.

The following table shows the change in the balance of contingent consideration payable for the three months ended March 31, 2019 and 2018, respectively:

| (in thousands) | Three Months Ended March 31, | |
|--|------------------------------|------------------|
| | 2019 | 2018 |
| Balance, beginning of the period | \$ 28,700 | \$ 25,400 |
| Payment of contingent consideration in stock | (9,316) | — |
| Changes in fair value during the period, included in Statement of Operations | 1,383 | 1,100 |
| Balance, end of the period | <u>\$ 20,767</u> | <u>\$ 26,500</u> |

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.

Derivative asset and liability

As of March 31, 2018, as further discussed in "— Note 5. Debt," 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 the Capped Call Confirmations that would not be able to be net share settled as a current derivative liability and as a current derivative asset, respectively. The fair value of the debt portion was determined using the discounted cash flow method of the income approach and the fair value of the Capped Call Confirmations was determined using the Black-Scholes model. The derivative asset and liability have been classified as Level 3 recurring as their 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 determined by the Company.

Note 9. Leases

The Company currently has operating and finance leases for office and research laboratory space, equipment and vehicles under agreements expiring at various dates through 2044. Certain of the Company's leases contain renewal options to extend the lease for up to 25 years.

For the three months ended March 31, 2019, lease expense was \$2.1 million and \$0.1 million for operating and finance leases, respectively. Cash paid for amounts included in the measurement of operating lease liabilities was \$1.4 million for the three months ended March 31, 2019. For the three months ended March 31, 2019, the Company recorded \$19.5 million of right-of-use assets obtained in exchange for new operating lease liabilities.

Commitments under finance leases are not significant.

Supplemental balance sheet information related to operating leases was as follows.

| (in thousands, except year and discount rate amounts) | March 31, 2019 |
|--|-----------------------|
| Operating lease ROU asset | \$ 36,208 |
| Current portion of the operating lease liabilities | \$ 2,421 |
| Non-current portion of the operating lease liabilities | 35,991 |
| Total operating lease liability | <u>\$ 38,412</u> |
| Weighted-average remaining lease terms (years) | 14.6 |
| Weighted-average discount rate | 12.8% |

At March 31, 2019, the future minimum lease payments were as follows:

| (in thousands) | Operating Leases |
|---|-------------------------|
| 2019 (excludes the three months ended March 31, 2019) | \$ 4,494 |
| 2020 | 9,776 |
| 2021 | 10,516 |
| 2022 | 10,418 |
| 2023 | 10,830 |
| Thereafter | 174,006 |
| Total lease payments | <u>\$ 220,040</u> |
| Less lease incentives | (28,939) |
| Less imputed interest | <u>(152,689)</u> |
| Total operating lease liability | <u>\$ 38,412</u> |

At December 31, 2018, the future minimum lease payments were as follows:

| (in thousands) | Operating Leases |
|-----------------------|-------------------------|
| 2019 | \$ 6,244 |
| 2020 | 4,063 |
| 2021 | 3,560 |
| 2022 | 3,371 |
| 2023 | 3,611 |
| Thereafter | 10,038 |
| Total lease payments | <u>\$ 30,887</u> |

Note 10. Basic and Diluted Net Loss per Common Share

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

| (in thousands, except per share amounts) | Three Months Ended March 31, | |
|--|------------------------------|-------------|
| | 2019 | 2018 |
| Numerator: | | |
| Net loss attributable to common stockholders | \$ (120,299) | \$ (49,916) |
| Denominator: | | |
| Weighted average common shares outstanding — basic and diluted | 213,519,287 | 175,977,700 |

Dilutive common stock equivalents would include the dilutive effect of common stock options, convertible debt units, RSUs and warrants for common stock equivalents. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

The table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method:

| (in thousands) | As of March 31, | |
|---|-----------------|---------------|
| | 2019 | 2018 |
| Options to purchase common stock | 18,331 | 16,176 |
| Convertible notes | 5,017 | 40,850 |
| Outstanding warrants, convertible to common stock | 2,554 | 3,110 |
| Unvested restricted stock units | 6,010 | 3,541 |
| Vested restricted stock units, unissued | 189 | 90 |
| Total number of potentially issuable shares | <u>32,101</u> | <u>63,767</u> |

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

Overview

We are a global patient-dedicated 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, we have a leading portfolio of therapies for lysosomal storage disorders ("LSDs").

The cornerstone of our portfolio is Galafold[®], (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. In February 2019, the U.S. Food and Drug Administration ("FDA") granted Breakthrough Therapy Designation to AT-GAA for the treatment of late onset Pompe disease. Our Chaperone-Advanced Replacement Therapy ("CHART[®]") platform technology is leveraged to develop novel products for Pompe disease and potentially other LSDs in the future.

With 14 new gene therapy programs, we have established a leading portfolio of medicines for people living with rare metabolic disorders. Through a license with NCH and collaboration with Penn, our pipeline includes gene therapy programs in rare, neurologic LSDs with lead programs in CLN6, CLN3, and CLN8 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD") and one additional undisclosed rare metabolic disorder.

We believe that our platform technologies and product pipeline uniquely positions us and drives our commitment to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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. We have made significant progress toward fulfilling our vision to build a leading global biotechnology company focused on rare metabolic diseases.

Highlights of our progress in the first quarter of 2019 include:

- *Commercial and regulatory success in Fabry disease.* During the three month ended March 31, 2019, Galafold[®] revenue totaled approximately \$34.0 million, an increase of \$17.4 million compared to the same period in the prior year. We continue to see strong momentum in new markets including in the U.S. and Japan, as well as in our more mature markets. In the countries we have been operating the longest, such as Germany and the United Kingdom, we see an increasing proportion of previously untreated patients come onto Galafold[®]. In the U.S., we continue to see a significant increase in patients from a growing and very wide prescriber base. Across all markets, we see a high rate of compliance and adherence to this oral treatment option.
- *Pompe clinical program milestones.* We reported positive data from a Phase 1/2 clinical study to evaluate Pompe disease patients treated with our novel treatment paradigm AT-GAA for up to 24 months. The U.S. FDA also granted breakthrough therapy designation ("BTD") for AT-GAA for the treatment of late-onset Pompe disease. We are currently enrolling a global pivotal study of AT-GAA (ATB200-03, also known as PROPEL) which is on track to enroll approximately 100 participants with late-onset Pompe disease at up to 90 global sites.
- *Pipeline Growth:* With 14 new gene therapy programs, we have established a leading portfolio of medicines for people living with rare metabolic disorders. Through our license with NCH, 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, CDKL5 deficiency disorder ("CDD") and one additional undisclosed rare metabolic disorder.

- *Manufacturing.* We successfully scaled up manufacturing of our Pompe biologic to commercial scale (1,000L) for our pivotal PROPEL study and commercial supply. Our supply agreement with WuXi Biologics and current capacity are expected 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 gene therapy programs.
- *Financial strength.* Total cash, cash equivalents and marketable securities of \$438.3 million at March 31, 2019 compared to \$504.2 million at December 31, 2018. The current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe and gene therapy program operations into at least mid-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

Galafold® (Migalastat HCl) for Fabry Disease

Our oral precision medicine Galafold® was granted accelerated approval by the 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. Galafold® was approved in the EU in May 2016 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 24 countries. We plan to continue to launch Galafold® in additional countries during 2019.

As an orally administered monotherapy, Galafold® 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. Galafold® 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.

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, which are not suitable for Galafold® 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.

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 initiated a global phase 3 clinical study (ATB200-03, or PROPEL) of AT-GAA in adult patients with late onset Pompe disease in 2018, with the first patient dosed in December 2018.

Our strategy is to enhance the body of clinical data for AT-GAA in ongoing clinical studies, including the pivotal study (PROPEL) to deliver this potential new therapy to as many people living with late onset Pompe disease as soon as possible. Based on regulatory feedback from both the U.S. FDA and the European Medicines Agency ("EMA"), the PROPEL study is expected to support approval for a broad indication, including ERT-switch and treatment-naïve patients, if the results are favorable.

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).

On February 5, 2019 we reported additional interim data from our clinical study ATB200-02 at the 15th Annual WORLDSymposium™. Highlights included safety and tolerability data in patients as well as pharmacodynamic "PD" data (muscle damage biomarker and disease substrate biomarker). To date, adverse events have been generally mild and transient. AT-GAA has resulted in a low rate of infusion-associated reactions ("IARs") following 1,110+ infusions (16 events of IARs in six patients). 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 up to month 24.

With regard to efficacy, muscle function improved in 16 out of 17 patients who have available data for up to 21 or 24 months. Mean 6MWT improved in both ERT-naïve and ERT-switch patients with continued benefit observed out to month 24. All 5 ERT-naïve patients showed increases in 6MWT distance at all time points out to month 21. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 63 meters at month 12 (n=5), and 55 meters at month 21 (n=5). 6MWT increased in 7/10, 9/10, and 8/8 ERT-switch patients at Months 6, 12, and 24 respectively. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 42 meters at month 12 (n=10), and 54 meters at month 24 (n=8). Other motor function tests generally showed mean improvements consistent with 6MWT distance out to month 21 or 24 in both ambulatory cohorts. Non-ambulatory ERT-switch patients showed improvements in upper extremity strength (which includes elbow and shoulder) from baseline to month 24, 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), +4.5% at month 12 (n=5), and +6.1% at month 21 (n=5). In ERT-switch patients mean absolute change in FVC was -1.2% at month 6 (n=9), -3.0% at month 12 (n=9), and -0.6% at month 24 (n=7). Overall, other pulmonary tests of maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation, were stable or increased in both ERT-naïve and ERT-switch patients.

Gene Therapy for Pompe Disease

As part of our long-term commitment to provide multiple solutions to address the significant unmet needs of the Pompe community, we are also advancing a next-generation gene therapy treatment for 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.

In April 2019, we presented initial preclinical data from our investigational adeno-associated viral ("AAV") gene therapy program for Pompe disease in mice. This initial preclinical study used a single high dose of AAV in the acid alpha-glucosidase ("GAA") knockout mice with either natural unmodified hGAA ("natural hGAA") or an Amicus/Penn engineered hGAA transgene with a Lysosomal-Targeting Cell receptor binding motif ("engineered hGAA"). The Amicus/Penn engineered hGAA AAV gene therapy demonstrated more uniform cellular uptake and lysosomal targeting compared to natural hGAA AAV gene therapy, as well as, robust glycogen reduction in all key tissues in Pompe disease that were assessed. In the central nervous system, the engineered hGAA AAV gene therapy showed robust glycogen reduction in neuronal cells, suggesting this may be an effective way to address neuronal aspects of Pompe disease. Natural hGAA AAV gene therapy did not show glycogen reduction in neuronal cells. This initial preclinical study provides initial validation for combining Amicus-engineered transgenes with Penn's AAV gene therapy technologies.

CDKL5 Deficiency Disorder ("CDD")

We are researching a potential first-in-class protein replacement therapy approach for CDD. In addition, through our collaboration with Penn, we are researching a gene therapy for CDD. 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 CDD. This 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.

Batten Disease Product Candidates

We are researching potential first-in-class gene therapies for multiple forms of Batten disease. Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses, or NCLs. In these diseases, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. Each gene is called CLN (ceroid lipofuscinosis, neuronal) and given a different number designation as its subtype. There are 13 known forms of Batten disease often referred to as CLN1-8; 10-14. The various types of Batten disease have similar features and symptoms but vary in severity and age of onset.

The two clinical stage gene therapies are in CLN3 and CLN6 Batten disease. The CLN6 Batten disease Phase 1/2 study completed target enrollment, with 12 patients receiving a single administration of adeno-associated virus serotype 9 AAV9-CLN6 gene therapy. We expect to report additional two-year data from CLN6 Batten disease Phase 1/2 study in 2019. In the CLN3 Batten disease study, a total of three patients were dosed in the low dose group with no serious adverse events after up to 5 months following a single administration of AAV9-CLN3 gene therapy.

Strategic Alliances and Arrangements

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 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 March 31, 2019 compared to March 31, 2018

The following table provides selected financial information for the Company:

| (in thousands) | Three Months Ended March 31, | | |
|---|------------------------------|-------------|-------------|
| | 2019 | 2018 | Change |
| Net product sales | \$ 34,046 | \$ 16,696 | \$ 17,350 |
| Cost of goods sold | 4,055 | 2,615 | 1,440 |
| Cost of goods sold as a percentage of net product sales | 11.9% | 15.7% | (3.8)% |
| Operating expenses: | | | |
| Research and development | 64,593 | 40,798 | 23,795 |
| Selling, general and administrative | 44,303 | 27,396 | 16,907 |
| Changes in fair value of contingent consideration payable | 1,383 | 1,100 | 283 |
| Depreciation and amortization | 991 | 969 | 22 |
| Other income (expense): | | | |
| Interest income | 2,639 | 1,737 | 902 |
| Interest expense | (6,454) | (4,488) | (1,966) |
| Change in fair value of derivatives | — | 4,861 | (4,861) |
| Loss on exchange of convertible notes | (36,123) | — | (36,123) |
| Other income | 1,086 | 2,764 | (1,678) |
| Income tax (expense) benefit | (168) | 1,392 | (1,560) |
| Net loss attributable to common stockholders | \$ (120,299) | \$ (49,916) | \$ (70,383) |

Net Product Sales. Net product sales increased \$17.4 million during the three months ended March 31, 2019 compared to the same period in the prior year. Galafold® was approved for sale in the U.S. and Japan in 2018.

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 11.9% during the three months ended March 31, 2019 compared to 15.7% 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 March 31, | |
|---|------------------------------|-----------|
| | 2019 | 2018 |
| Projects | | |
| Third party direct project expenses | | |
| Migalastat (Fabry Disease) | \$ 4,356 | \$ 3,708 |
| AT-GAA (Pompe Disease) | 28,286 | 15,095 |
| Gene therapy programs | 2,192 | — |
| Pre-clinical and other programs | 460 | 658 |
| Total third party direct project expenses | 35,294 | 19,461 |
| Other project costs | | |
| Personnel costs | 19,634 | 14,949 |
| Other costs | 9,665 | 6,388 |
| Total other project costs | 29,299 | 21,337 |
| Total research and development costs | \$ 64,593 | \$ 40,798 |

The increase in research and development costs was primarily due to increases in Pompe's clinical research and manufacturing costs with the advancement and enrollment of clinical studies, an increase in gene therapy programs driven by the pipeline growth in gene therapy programs in the second half of 2018. There were also increases in personnel and other costs with the advancement and enrollment of clinical studies and investments in manufacturing.

Selling, General and Administrative Expense. Selling, general and administrative increased \$16.9 million primarily due to the expanded geographic scope of the ongoing commercial launch of Galafold® and related operational costs of our global business, including establishing commercial organizations and related teams in the U.S and Japan.

Change in Fair Value of Derivatives. Subsequent to the underwritten public offerings in February 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 was determined and subsequent changes to the fair value of the derivatives were recorded through earnings on the Company's consolidated statement of operations resulting in a change in fair value of derivatives for the three months ended March 31, 2018 of \$4.9 million.

Loss on Exchange of Convertible Notes. During the first quarter of 2019, the Company entered into separate, privately negotiated exchange agreements with a limited number of holders (the "Holders") of the Convertible Notes. As a result of this exchange, the Company recognized a loss on exchange of debt of \$36.1 million in the consolidated statement of operations, and \$190.4 million in additional paid-in-capital and common stock of \$0.4 million in the consolidated balance sheets for the three months ended March 31, 2019.

Other Income. The \$1.7 million decrease in other income was primarily due to unrealized losses on foreign exchange transactions.

Income Tax (Expense) Benefit. The income tax expense for the three months ended March 31, 2019 is related to the current period provision for income taxes. 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 March 31, 2018 was \$1.4 million and was primarily due to a discrete tax item.

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 first quarter of 2019, we terminated the proportion of the Capped Call Confirmations related to the exchange of the Convertible Notes during the first quarter of 2019 for cash proceeds of approximately \$14.6 million. We also entered into separate, privately negotiated exchange agreements with a limited number of holders (the "Holders") of the Convertible Notes. Under the terms of the exchange agreements (the "Exchange Agreements"), the Holders agreed to exchange an aggregate principal amount of approximately \$219.3 million of Convertible Notes held by them in exchange for an aggregate of approximately 39.0 million shares of our common stock, par value \$0.01 per share.

Cash flow discussion

As of March 31, 2019, we had cash, cash equivalents and marketable securities of \$438.3 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 3. 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 three months ended March 31, 2019 was \$78.5 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2019 of \$120.3 million and the net change in operating assets and liabilities of \$11.5 million. The change in operating assets was primarily due to increases in accounts receivable by \$1.0 million, partially offset by decrease in prepaid and other current assets of \$3.1 million for spending to support commercial activities for Galafold® launch and decrease in inventory of \$0.3 million due to increased commercial sales of Galafold®. The net cash used in operations was also impacted by decrease in accounts payable and accrued expenses of \$8.9 million, mainly related to program expenses and support for the commercial launch of Galafold®, and a decrease in deferred reimbursement of \$1.5 million due to payment of a milestone.

Net cash used in operations for the three months ended March 31, 2018 was \$49.5 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2018 of \$49.9 million and the decrease in operating assets of \$6.2 million. The change in operating assets was primarily due to decrease in prepaid expenses and other current assets of \$10.9 million for spending to support commercial activities for Galafold® launch, partially offset by an increase in inventory of \$3.3 million and an increase in accounts receivable by \$1.0 million due to commercial sales of Galafold®. The net cash used in operations was also impacted by decrease in accounts payable and accrued expenses of \$10.3 million related to program expenses and support for the commercial launch of Galafold®.

Net Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities for the three months ended March 31, 2019 was \$80.1 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash provided by investing activities reflects \$135.2 million for the sale and redemption of marketable securities, partially offset by \$52.2 million for the purchase of marketable securities, and \$2.9 million for the acquisition of property and equipment.

Net cash used in investing activities for the three months ended March 31, 2018 was \$182.6 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 \$303.0 million for the purchase of marketable securities, and \$0.8 million for the acquisition of property and equipment, partially offset by \$121.2 million for the sale and redemption of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2019 was \$17.4 million. Net cash provided by financing activities primarily reflects \$14.6 million from partial termination of capped call and \$4.8 million from the exercise of stock options and warrants, partially offset by \$1.9 million from the purchase of vested restricted stock units.

Net cash provided by financing activities for the three months ended March 31, 2018 was \$296.6 million. Net cash provided by financing activities primarily reflects \$294.6 million from the issuance of common stock, net of issuance costs, \$1.9 million from the vesting of RSUs and \$0.07 million in payments on capital lease arrangements, partially offset by \$4.0 million received from the exercise of stock options.

Funding Requirements

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

- the progress and results of our preclinical and clinical trials of our drug candidates and gene therapy candidates;
- the cost of manufacturing drug and gene therapy supply for our clinical and preclinical studies, including the significant cost of manufacturing Pompe ERT and gene therapies;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of LSDs and gene therapies for the treatment of rare genetic metabolic diseases;

- the future results of on-going preclinical research and subsequent clinical trials for CDD, 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 Galafold® ("migalastat HCl");
- our ability to manufacture or supply sufficient clinical or commercial products;
- our ability to obtain reimbursement for Galafold®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold®;
- our ability to obtain market acceptance of Galafold®;
- 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;
- our ability to adjust to changes in European and United Kingdom markets as the United Kingdom leaves the European Union; and
- fluctuations in foreign currency exchange rates; and changes in accounting standards.

While we continue to generate revenue from product sales, 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 mid-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

Celenex - With our acquisition of Celenex in 2018, we 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.

NCH - 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.

Penn - Under our collaboration agreement with Penn, 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.

MSSM - 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.

GSK - 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 three months ended March 31, 2019, under the MSSM and GSK license and collaboration agreements, we paid \$4.5 million in royalties and \$1.5 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 three months ended March 31, 2019 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, 2018, except as it relates to the adoption of ASU 2016-02, Leases (Topic 842), which is described below.

Leases

In the ordinary course of business, the Company enters into lease agreements for office space as well as leases for certain property and equipment. The leases have varying terms and expirations and have provisions to extend or renew the lease agreement, among other terms and conditions, as negotiated. The Company determines if an arrangement is a lease at inception. Operating and finance leases are included in right-of-use ("ROU") assets and lease liabilities on the Company's consolidated balance sheets.

ROU assets represent the Company's right to control the use of an explicitly or implicitly identified fixed asset for a period of time and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term.

Lease payments included in the measurement of the lease liability are comprised of fixed payments. Variable lease payments are excluded from the ROU assets and lease liabilities and are recognized in the period in which the obligation for those payments are incurred. Variable lease payments are presented in the Company's consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all underlying asset categories.

A lessee is required to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments.

The lease term for all the Company's leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Leases with an initial term of 12 months or less are not recorded on the balance sheet. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. For additional information, see "—Note 9. Leases" and "—Note 2. Summary of Significant Accounting Policies, Recent Accounting Developments - Guidance Adopted in 2019."

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 \$1.2 million as of March 31, 2019. 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.

We are exposed to interest rate risk with respect to variable rate debt. At December 31, 2018, we had \$150 million aggregate principal amount of variable rate debt through our Senior Secured Term Loan. We do not currently hedge our variable interest rate debt. The average variable interest rate for our variable rate debt as of March 31, 2019 was 10.1%. A hypothetical 100 basis point increase or decrease in the average interest rate on our variable rate debt would not result in a material change in the interest expense.

We face foreign exchange risk as a result of entering into transactions denominated in currencies other than U.S. dollars. We are not currently engaged in any foreign currency hedging activities. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, and net product sales denominated in foreign currencies. Both positive and negative impacts to our international product sales from movements in foreign currency exchange rates may be partially mitigated by the natural, opposite impact that foreign currency exchange rates have on our international operating expenses. A hypothetical 10% change in foreign exchange rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

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, 2018. There have been no material changes in our financial instrument portfolio or market risk exposures since our fiscal year ended December 31, 2018.

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

We are not currently a party to any legal proceedings.

ITEM 1A. RISK FACTORS

There have been no material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018.

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

None.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

| Exhibit Number | Description |
|----------------|---|
| 10.1 | Form of Call Option Partial Termination Agreement |
| 31.1 | Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 31.2 | Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 32.1 | Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 101 | The following financial information from this Quarterly Report on Form 10-Q for the three months ended March 31, 2019, 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 |

PARTIAL TERMINATION AGREEMENT

This PARTIAL TERMINATION AGREEMENT (this "**Termination Agreement**") with respect to the Call Option Confirmation (as defined below) is made as of [●] between [●] ("**Dealer**") and Amicus Therapeutics, Inc. ("**Counterparty**"), a Delaware corporation.

WHEREAS, Counterparty issued \$250,000,000 principal amount of 3.00% Convertible Senior Notes due 2023 (the "**Convertible Notes**") pursuant to an Indenture dated as of December 21, 2016 between Counterparty and Wilmington Trust, National Association, as trustee;

WHEREAS, Dealer and Counterparty are parties to the base capped call option transaction (as amended, modified or supplemented, the "**Base Call Option Transaction**") evidenced by the letter agreement between Dealer and Counterparty, dated as of December 15, 2016 (as amended, modified or supplemented, the "**Base Call Option Confirmation**"), and the additional capped call option transaction (as amended, modified or supplemented, the "**Additional Call Option Transaction**" and, together with the Base Call Option Transaction, the "**Call Option Transactions**") evidenced by the letter agreement between Dealer and Counterparty, dated as of December 19, 2016 (as amended, modified or supplemented, the "**Additional Call Option Confirmation**" and, together with the Base Call Option Confirmation, the "**Call Option Confirmations**"); and

WHEREAS, as a result of a repurchase by Counterparty of [●] Convertible Notes in \$1,000 principal amount denominations, a Repayment Event and consequently an Additional Termination Event occurred under the Call Option Transactions;

WHEREAS, in lieu of partial terminations under the Call Option Transactions Counterparty has requested the full termination of the Additional Call Option Transaction and partial termination of the Base Call Option Transaction.

NOW, THEREFORE, in consideration of their mutual covenants contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto, intending to be legally bound, hereby mutually covenant and agree as follows:

- I. Defined Terms. Any capitalized term not otherwise defined herein shall have the meaning set forth for such term in the Call Option Confirmations.
 2. Termination of Transactions and Confirmations. Notwithstanding anything to the contrary in the Call Option Confirmations, Counterparty and Dealer agree that, effective on the date hereof, (i) the Additional Call Option Transaction shall automatically terminate and all of the respective rights and obligations of the parties under the Additional Call Option Confirmation shall be terminated, cancelled and extinguished, (ii) the Base Call Option Confirmation shall be amended solely to reduce the Number of Options thereunder by [●] such that the Number of Options thereunder after giving effect to such amendment shall be [●] and the Base Call Option Confirmation, as so amended, shall remain in full force and effect and (iii) in consideration of the foregoing, Dealer shall pay to Counterparty, in immediately available funds to the account specified below, cash in US Dollars in an amount equal to the Cash Settlement Amount no later than 4:00 p.m. (New York City time) on the first Clearance System Business Day following the conclusion of the Hedge Unwind Period. "**Cash Settlement Amount**" and "**Hedge Unwind Period**" shall have the meanings assigned to such terms in Exhibit A hereto.
 3. Representations and Warranties of Counterparty. Counterparty represents and warrants to Dealer on the date hereof that:
 - (a) it is duly organized and validly existing under the laws of the jurisdiction of its organization or incorporation and, if relevant under such laws, in good standing;
 - (b) it has the power to execute this Termination Agreement and any other documentation relating to this Termination Agreement, to deliver this Termination Agreement and each such other document relating thereto (if any) and to perform its obligations hereunder and thereunder (as applicable) and has taken all necessary action to authorize such execution, delivery and performance;
-

(c) such execution, delivery and performance do not violate or conflict with any law applicable to it, any provision of its constitutional documents, any order or judgment of any court or other agency of government applicable to it or any of its assets or any contractual restriction binding on or affecting it or any of its assets;

(d) all governmental and other consents that are required to have been obtained by it with respect to this Termination Agreement have been obtained and are in full force and effect and all conditions of any such consents have been complied with;

(e) its obligations under this Termination Agreement constitute its legal, valid and binding obligations, enforceable in accordance with their respective terms (subject to applicable bankruptcy, reorganization, insolvency, moratorium or similar laws affecting creditors' rights generally and subject, as to enforceability, to equitable principles of general application (regardless of whether enforcement is sought in a proceeding in equity or at law));

(t) it is not aware of any material nonpublic information regarding Counterparty or the Shares;

(g) it (A) is capable of evaluating investment risks independently, both in general and with regard to all transactions and investment strategies involving a security or securities; (B) will exercise independent judgment in evaluating the recommendations of any broker-dealer or its associated persons, unless it has otherwise notified the broker-dealer in writing; and (C) has total assets of at least \$50 million;

(h) it is not entering into this Termination Agreement to create actual or apparent trading activity in the Shares (or any security convertible into or exchangeable for the Shares) or to raise or depress or otherwise manipulate the price of the Shares (or any security convertible into or exchangeable for the Shares) or otherwise in violation of the Exchange Act; and

(i) on the date hereof, it remains a party to the Call Option Transactions to the full extent as on the date of execution thereof and it has not assigned, purported to assign or made any attempt to assign, any interest in the Call Option Transactions to any third party.

4. Representations and Warranties of Dealer. Dealer represents and warrants to Counterparty on the date hereof that:

(a) it is duly organized and validly existing under the laws of the jurisdiction of its organization or incorporation and, if relevant under such laws, in good standing;

(b) it has the power to execute this Termination Agreement and any other documentation relating to this Termination Agreement to which it is a party, to deliver this Termination Agreement and each such other document (if any) and to perform its obligations hereunder and thereunder (as applicable) and has taken all necessary action to authorize such execution, delivery and performance;

(c) such execution, delivery and performance do not violate or conflict with any law applicable to it, any provision of its constitutional documents, any order or judgment of any court or other agency of government applicable to it or any of its assets or any contractual restriction binding on or affecting it or any of its assets;

(d) all governmental and other consents that are required to have been obtained by it with respect to this Termination Agreement have been obtained and are in full force and effect and all conditions of any such consents have been complied with;

(e) its obligations under this Termination Agreement constitute its legal, valid and binding obligations, enforceable in accordance with their respective terms (subject to applicable bankruptcy, reorganization, insolvency, moratorium or similar laws affecting creditors' rights generally and subject, as to enforceability, to equitable principles of general application (regardless of whether enforcement is sought in a proceeding in equity or at law)); and

(t) on the date hereof, it remains a party to the Call Option Transactions to the full extent as on the date of execution thereof and it has not assigned, purported to assign or made any attempt to assign, any interest in the Call Option Transactions to any third party.

5. Notices. For purposes of this Termination Agreement, the addresses for notices or communications to the parties shall be:

(a) Counterparty:

Amicus Therapeutics, Inc. 1 Cedar Brook Drive Cranbury, NJ 08512

Attention: [●]

Telephone No.: [●]

Email: [●]

With a copy to:

Attention: [●]

Telephone No.: [●]

Email: [●]

(b) Dealer:

For purpose of Giving Notice:

To: [●]

Attention: [●]

Email: [●]

For Trade Affirmations and Settlements:

To: [●]

Attention: [●]

Email: [●]

For Trade Confirmations:

To: [●]

Attention: [●]

Email: [●]

6. Account Details. For purposes of this Termination Agreement, the account for payments to Counterparty shall be:

Bank: ABA#:

Acct No.:

Beneficiary:

SWIFT ID:

[●]

[●]

[●]

[●]

[●]

7. Disclosure. Notwithstanding anything provided in this Termination Agreement or the Call Option Confirmations, effective from the date of commencement of discussions concerning the Call Option Transactions, Counterparty and each of its employees, representatives, or other agents may disclose to any and all persons, without limitation of any kind, the tax treatment and tax structure of the Call Option Transactions and this Termination Agreement and all materials of any kind (including opinions or other tax analyses) that are provided to Counterparty relating to such tax treatment and tax structure.

8. No Reliance, etc. Counterparty hereby confirms that it has relied on the advice of its own counsel and other advisors (to the extent it deems appropriate) with respect to any legal, tax, accounting, or regulatory consequences of this Termination Agreement, that it has not relied on Dealer or its affiliates in any respect in connection therewith, and that it will not hold Dealer or its affiliates accountable for any such consequences.

9. Role of Agent. Each party agrees and acknowledges that (i) Dealer has acted solely as agent and not as principal with respect to this Termination Agreement and (ii) Dealer has no obligation or liability, by way of guaranty, endorsement or otherwise, in any manner in respect of this Termination Agreement (including, if applicable, in respect of the settlement thereof). Each party agrees it will look solely to the other party (or any guarantor in respect thereof) for performance of such other party's obligations under this Termination Agreement. Dealer is authorized to act as agent for Dealer.

10. Designation by Dealer. Notwithstanding any other provision in this Termination Agreement to the contrary requiring or allowing Dealer to purchase, sell, receive or deliver any Shares or other securities to or from Counterparty, Dealer may designate any of its affiliates to purchase, sell, receive or deliver such shares or other securities and otherwise to perform Dealer obligations in respect of the transactions contemplated by this Termination Agreement and any such designee may assume such obligations. Dealer shall be discharged of its obligations to Counterparty to the extent of any such performance.

11. Governing Law. THIS TERMINATION AGREEMENT AND ALL MATTERS ARISING IN CONNECTION WITH THIS TERMINATION AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED AND ENFORCED IN ACCORDANCE WITH, THE LAWS OF THE STATE OF NEW YORK (WITHOUT REFERENCE TO ITS CHOICE OF LAW DOCTRINE, OTHER THAN TITLE 14 OF THE NEW YORK GENERAL OBLIGATIONS LAW).

12. Waiver of Trial by Jury. EACH OF COUNTERPARTY AND DEALER HEREBY IRREVOCABLY WAIVES (ON ITS OWN BEHALF AND, TO THE EXTENT PERMITTED BY APPLICABLE LAW, ON BEHALF OF ITS STOCKHOLDERS) ALL RIGHT TO TRIAL BY JURY IN ANY ACTION, PROCEEDING OR COUNTERCLAIM (WHETHER BASED ON CONTRACT, TORT OR OTHERWISE) ARISING OUT OF OR RELATING TO THE TRANSACTION OR THE ACTIONS OF DEALER OR ITS AFFILIATES IN THE NEGOTIATION, PERFORMANCE OR ENFORCEMENT HEREOF.

13. Amendment. This Termination Agreement may not be modified, amended or supplemented, except in a written instrument signed by each party hereto.

14. Counterparts. This Termination Agreement may be executed in several counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument.

15. Agreements and Acknowledgements Regarding Hedging. Counterparty acknowledges and agrees that:

(a) on or following the date hereof, Dealer and its Affiliates may buy or sell Shares or other securities or buy or sell options or futures contracts or enter into swaps or other derivative securities in order to adjust its hedge position with respect to this Termination Agreement;

(b) Dealer and its Affiliates also may be active in the market for Shares other than in connection with hedging activities in relation to this Termination Agreement;

(c) Dealer shall make its own determination as to whether, when or in what manner any hedging or market activities in Counterparty's securities shall be conducted and shall do so in a manner that it deems appropriate to hedge its price and market risk; and

(d) any market activities of Dealer and its Affiliates with respect to Shares may affect the market price and volatility of Shares each in a manner that may be adverse to Counterparty.

16. No Other Changes. Except as expressly set forth herein, all of the terms and conditions of the Call Option Confirmations shall remain in full force and effect and are hereby confirmed in all respects.

[The remainder of page intentionally left blank]

IN WITNESS WHEREOF the parties have executed this Termination Agreement with effect from the date specified on the first page of this Termination Agreement.

DEALER

By: ____
Name : Title:

Amicus Therapeutics, Inc.

By: ____
Name : Title:

EXHIBIT A

| VWAP Price | Cash Settlement Amount |
|------------|------------------------|
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |
| \$[•] | \$[•] |

"Cash Settlement Amount" means the amount set forth in the table above opposite the applicable VWAP Price (as defined below). If the VWAP Price is not specified on the table above, the Cash Settlement Amount shall be the straight-line interpolation between the VWAP Prices or extrapolation from the VWAP Prices (as the case may be) specified on the table above. Dealer shall notify Counterparty of the applicable Cash Settlement Amount as soon as reasonably practicable after 5:00pm (New York City time) on the last Scheduled Trading Day of the Hedge Unwind Period.

"VWAP Price" means the arithmetic average of the Daily Prices for all Exchange Business Days in the Hedge Unwind Period, as determined by Dealer in good faith and a commercially reasonable manner.

"Daily Price" means, for any Exchange Business Day, the per Share volume-weighted average price as displayed under the heading "Bloomberg VWAP" on Bloomberg Page "FOLD <Equity> AQR" (or any successor thereto) for each such Exchange Business Day in respect of the period from the scheduled opening time of the Exchange to the Scheduled Closing Time of the Exchange on such Exchange Business Day (or, if such volume-weighted average price is unavailable at such time, the market value of one Share on such Exchange Business Day, as determined by the Calculation Agent using, if practicable, a volume-weighted average method). The VWAP Price will be determined without regard to after-hours trading or any other trading outside of the regular trading session trading hours.

"Hedge Unwind Period" means the Scheduled Trading Day occurring on [•]; *provided* that, notwithstanding anything to the contrary in this Termination Agreement, Dealer may postpone such Scheduled Trading Day or extend the Hedge Unwind Period by one or more Scheduled Trading Days if Dealer determines, in its reasonable discretion, that such postponement or extension is reasonably necessary or appropriate to preserve Dealer's hedge unwind activity hereunder in light of existing liquidity conditions or to enable Dealer (or any of its affiliates) to effect transactions .of Shares in connection with its hedge unwind activity hereunder in a manner that would, if Dealer (or such affiliate) were Counterparty or an affiliated purchaser of Counterparty; be in compliance with applicable legal, regulatory or self-regulatory requirements, or with related policies and procedures applicable to Dealer.

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

I, John F. Crowley, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2019

/s/ John F. Crowley

John F. Crowley

Chairman and Chief Executive Officer

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

I, Daphne Quimi, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2019

/s/ Daphne Quimi

Daphne Quimi
Chief Financial Officer

