

**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, 2020

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 27, 2020 was 257,664,403 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended March 31, 2020

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

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks, uncertainties, and assumptions. Forward-looking statements are all statements, other than statements of historical facts, that discuss our current expectation and projections relating to our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans, and objectives of management. These statements may be preceded by, followed by, or include the words "aim," "anticipate," "believe," "can," "could," "estimate," "expect," "forecast," "intend," "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 and gene therapy candidates, including but not limited to AT-GAA, CLN6 and CLN3;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the cost of manufacturing Pompe Enzyme Replacement Therapy ("ERT" or "ATB200") 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 a pharmacological chaperone co-administered with ERT for the treatment of Pompe disease ("AT-GAA") 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, Pompe gene therapy, Fabry gene therapy, Niemann- Pick Type C ("NPC"), Mucopolysaccharidosis Type IIIB ("MPSIIIB") and next generation Mucopolysaccharidosis Type IIIA ("MPSIIIA"), including our ability to obtain regulatory approvals and commercialize these gene therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- any changes in regulatory standards relating to the 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, including Galafold®, AT-GAA and our gene therapy candidates;
- 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, partnerships or other similar arrangements and to obtain milestone, royalty, or other payments from any such collaborators;
- our ability to adjust to changes in the European and United Kingdom markets in the wake of the United Kingdom leaving the European Union;

- the extent to which our business could be adversely impacted by the effects of the novel coronavirus ("COVID-19") outbreak, including due to actions by us, governments, our customers or suppliers or other third parties to control the spread of COVID-19, or by other health epidemics or pandemics;
- 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, 2019, 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, 2019 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, 2020 | December 31, 2019 |
|--|-------------------|-------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 123,231 | \$ 142,837 |
| Investments in marketable securities | 215,642 | 309,903 |
| Accounts receivable | 40,555 | 33,284 |
| Inventories | 12,831 | 14,041 |
| Prepaid expenses and other current assets | 15,690 | 20,008 |
| Total current assets | 407,949 | 520,073 |
| Operating lease right-of-use assets, less accumulated amortization of \$6,260 and \$5,342 at March 31, 2020 and December 31, 2019, respectively | 32,501 | 33,315 |
| Property and equipment, less accumulated depreciation of \$19,260 and \$17,604 at March 31, 2020 and December 31, 2019, respectively | 47,688 | 47,705 |
| In-process research & development | 23,000 | 23,000 |
| Goodwill | 197,797 | 197,797 |
| Other non-current assets | 29,414 | 28,317 |
| Total Assets | \$ 738,349 | \$ 850,207 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 16,239 | \$ 21,722 |
| Accrued expenses and other current liabilities | 66,722 | 98,651 |
| Deferred reimbursements | 1,250 | 1,250 |
| Operating lease liabilities | 7,503 | 7,189 |
| Total current liabilities | 91,714 | 128,812 |
| Deferred reimbursements | 8,906 | 8,906 |
| Convertible notes | 2,167 | 2,131 |
| Senior secured term loan | 147,569 | 147,374 |
| Contingent consideration payable | 23,612 | 22,681 |
| Deferred income taxes | 5,051 | 5,051 |
| Operating lease liabilities | 52,522 | 53,531 |
| Other non-current liabilities | 4,214 | 5,296 |
| Total liabilities | 335,755 | 373,782 |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Common stock, \$0.01 par value, 500,000,000 shares authorized, 257,449,955 and 255,417,869 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively | 2,607 | 2,598 |
| Additional paid-in capital | 2,238,346 | 2,227,225 |
| Accumulated other comprehensive loss: | | |
| Foreign currency translation adjustment | 6,981 | 2,785 |
| Unrealized (loss) gain on available-for-sale securities | (169) | 40 |
| Warrants | 12,387 | 12,387 |
| Accumulated deficit | (1,857,558) | (1,768,610) |
| Total stockholders' equity | 402,594 | 476,425 |
| Total Liabilities and Stockholders' Equity | \$ 738,349 | \$ 850,207 |

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, | |
|---|------------------------------|---------------------|
| | 2020 | 2019 |
| Net product sales | \$ 60,525 | \$ 34,046 |
| Cost of goods sold | 6,552 | 4,055 |
| Gross profit | 53,973 | 29,991 |
| Operating expenses: | | |
| Research and development | 89,120 | 64,593 |
| Selling, general, and administrative | 40,215 | 44,303 |
| Changes in fair value of contingent consideration payable | 931 | 1,383 |
| Depreciation and amortization | 1,764 | 991 |
| Total operating expenses | 132,030 | 111,270 |
| Loss from operations | (78,057) | (81,279) |
| Other income (expense): | | |
| Interest income | 1,515 | 2,639 |
| Interest expense | (3,729) | (6,454) |
| Loss on exchange of convertible notes | — | (36,123) |
| Other (expense) income | (8,316) | 1,086 |
| Loss before income tax | (88,587) | (120,131) |
| Income tax expense | (361) | (168) |
| Net loss attributable to common stockholders | \$ (88,948) | \$ (120,299) |
| Net loss attributable to common stockholders per common share — basic and diluted | \$ (0.35) | \$ (0.56) |
| Weighted-average common shares outstanding — basic and diluted | 256,968,248 | 213,519,287 |

See accompanying Notes to Consolidated Financial Statements

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

| | Three Months Ended March 31, | |
|--|------------------------------|--------------|
| | 2020 | 2019 |
| Net loss | \$ (88,948) | \$ (120,299) |
| Other comprehensive gain (loss): | | |
| Foreign currency translation adjustment gain (loss), net of tax impact of \$1,536 and \$0, respectively | 4,196 | (1,804) |
| Unrealized (loss) gain on available-for-sale securities, net of tax impact of \$(56) and \$0, respectively | (209) | 584 |
| Other comprehensive income | \$ 3,987 | \$ (1,220) |
| Comprehensive loss | \$ (84,961) | \$ (121,519) |

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, 2020

| | Common Stock | | Additional Paid-In Capital | Warrants | Other Comprehensive Gain (Loss) | Accumulated Deficit | Total Stockholders' Equity |
|--|--------------|----------|----------------------------------|-----------|---------------------------------------|------------------------|----------------------------------|
| | Shares | Amount | | | | | |
| Balance at December 31, 2019 | 255,417,869 | \$ 2,598 | \$ 2,227,225 | \$ 12,387 | \$ 2,825 | \$ (1,768,610) | \$ 476,425 |
| Stock issued from exercise of stock options, net | 958,947 | 9 | 6,068 | — | — | — | 6,077 |
| Restricted stock tax vesting | 1,073,139 | — | (7,543) | — | — | — | (7,543) |
| Stock-based compensation | — | — | 12,596 | — | — | — | 12,596 |
| Unrealized holding loss on available-for-sale securities | — | — | — | — | (209) | — | (209) |
| Foreign currency translation adjustment | — | — | — | — | 4,196 | — | 4,196 |
| Net loss | — | — | — | — | — | (88,948) | (88,948) |
| Balance at March 31, 2020 | 257,449,955 | \$ 2,607 | \$ 2,238,346 | \$ 12,387 | \$ 6,812 | \$ (1,857,558) | \$ 402,594 |

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 |

See accompanying Notes to Consolidated Financial Statements

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

| | Three Months Ended March 31, | |
|--|------------------------------|------------------|
| | 2020 | 2019 |
| Operating activities | | |
| Net loss | \$ (88,948) | \$ (120,299) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Amortization of debt discount and deferred financing | 231 | 1,649 |
| Depreciation and amortization | 1,764 | 991 |
| Stock-based compensation | 12,596 | 12,744 |
| Loss on exchange of convertible debt | — | 36,123 |
| Non-cash changes in the fair value of contingent consideration payable | 931 | 1,383 |
| Foreign currency remeasurement loss | 10,662 | 325 |
| Changes in operating assets and liabilities: | | |
| Accounts receivable | (8,358) | (1,001) |
| Inventories | (251) | 345 |
| Prepaid expenses and other current assets | 4,016 | 3,059 |
| Accounts payable and accrued expenses | (35,251) | (8,858) |
| Other non-current assets and liabilities | (5,334) | (3,507) |
| Deferred reimbursements | — | (1,500) |
| Net cash used in operating activities | \$ (107,942) | \$ (78,546) |
| Investing activities | | |
| Sale and redemption of marketable securities | 106,140 | 135,187 |
| Purchases of marketable securities | (12,088) | (52,178) |
| Capital expenditures | (806) | (2,944) |
| Net cash provided by investing activities | \$ 93,246 | \$ 80,065 |
| Financing activities | | |
| Payment of finance leases | (21) | (75) |
| Purchase of vested restricted stock units | (7,543) | (1,938) |
| Proceeds from termination of capped call confirmations | — | 14,632 |
| Proceeds from exercise of stock options | 6,077 | 3,953 |
| Proceeds of exercise of warrants | — | 812 |
| Net cash (used in) provided by financing activities | \$ (1,487) | \$ 17,384 |
| Effect of exchange rate changes on cash, cash equivalents, and restricted cash | \$ (4,265) | \$ (2,350) |
| Net (decrease) increase in cash, cash equivalents, and restricted cash at the end of the period | (20,448) | 16,553 |
| Cash, cash equivalents, and restricted cash at beginning of period | 146,341 | 82,375 |
| Cash, cash equivalents, and restricted cash at the end of period | \$ 125,893 | \$ 98,928 |
| Supplemental disclosures of cash flow information | | |
| Tenant improvements paid through lease incentives | \$ 254 | \$ — |
| Cash paid during the period for interest | \$ 3,693 | \$ 4,846 |
| Contingent consideration paid in shares | \$ — | \$ 9,316 |
| Capital expenditures unpaid at the end of period | \$ 589 | \$ — |

See accompanying Notes to Consolidated Financial Statements

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

Note 1. Description of Business

Amicus Therapeutics, Inc. (the "Company") is a global, patient-dedicated biotechnology company focused on discovering, developing, and delivering novel medicines for rare diseases. The Company has a portfolio of product opportunities led by the first, oral monotherapy for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease in the clinic, and an industry leading rare disease gene therapy portfolio.

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 ("E.U."), United Kingdom ("U.K."), and Japan, with multiple additional approvals granted and applications pending in several other geographies around the world.

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 ("BTD") to AT-GAA for the treatment of late onset Pompe disease. In the first quarter of 2020, the British Medicines and Healthcare Products Regulatory Agency issued a Promising Innovative Medicine designation for AT-GAA for the treatment of late-onset Pompe disease.

The Company has established an industry leading gene therapy portfolio of potential therapies for people living with rare metabolic diseases, through a license with Nationwide Children's Hospital ("Nationwide Children's") and an expanded collaboration with the University of Pennsylvania ("Penn"). The Company's pipeline includes gene therapy programs in rare, neurologic lysosomal disorders ("LDs"), specifically: CLN6, CLN3, and CLN1 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD"), Niemann-Pick Type C ("NPC"), Mucopolysaccharidosis Type IIIB ("MPSIIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPSIIIA"). This expanded collaboration with Penn also provides the Company with exclusive disease-specific access and option rights to develop potentially disruptive new gene therapy platform technologies and programs for most LDs and a broader portfolio of more prevalent rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy, and select other muscular dystrophies. In the first quarter of 2020, the FDA granted Fast Track designation to the CLN3 Batten disease gene therapy, AT-GTX-502, for the treatment of pediatric patients less than 18 years of age.

The Company's operations have not yet been significantly impacted from the novel coronavirus ("COVID-19") pandemic. The Company has maintained operations in all geographies, secured its global supply chain for its commercial and clinical products, and maintained its clinical trials, with minimal disruption. The Company believes its ability to continue to operate without any significant disruptions will depend on the continued health of its employees, the ongoing demand for Galafold[®] and the continued operation of its global supply chain. The Company has continued to provide uninterrupted access to medicines for those in need of treatment, while prioritizing the health and safety of its global workforce. However, the Company's results of operations in future periods may be negatively impacted by unknown future impacts from the COVID-19 pandemic.

The Company had an accumulated deficit of \$1.9 billion as of March 31, 2020 and anticipates incurring losses through the fiscal year ending December 31, 2020 and beyond. The Company has historically funded its operations through stock offerings, debt issuances, Galafold[®] revenues, collaborations, and other financing arrangements.

The current cash position, including expected Galafold[®] revenues, is sufficient to fund ongoing Fabry, Pompe, and gene therapy program operations well into the second half of 2022. 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 U.S. generally accepted accounting principles ("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, 2019. 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, 2019.

Consolidation

The Consolidated Financial Statements include the accounts of the Company and its subsidiaries. Intercompany accounts and transactions are 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.

Additionally, the Company assessed the impact COVID-19 pandemic has had on its operations and financial results as of March 31, 2020 and through the issuance of this report. The Company's analysis was informed by the facts and circumstances as they were known to the Company. This assessment considered the impact COVID-19 may have on financial estimates and assumptions that affect the reported amounts of assets and liabilities and revenue and expenses.

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 Sheets. 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 Sheets.

Concentration of Credit Risk

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash, 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 its cash, cash equivalents, or 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, 2020 have arisen from product sales primarily in Europe and the U.S. The Company will periodically assess the financial strength of its customers and the geographic economic environments and conditions 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. As of March 31, 2020, the Company recorded an allowance for doubtful accounts of \$0.1 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 the sale 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, | |
|-------------------------|------------------------------|-----------|
| | 2020 | 2019 |
| U.S. | \$ 17,772 | \$ 9,068 |
| Ex-U.S. | 42,753 | 24,978 |
| Total net product sales | \$ 60,525 | \$ 34,046 |

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.

Leases

The Company primarily enters into lease agreements for office space, equipment, and vehicles. The leases have varying terms, some of which could include options to renew, extend, and early terminate. The Company determines if an arrangement is a lease at contract inception. Operating leases are included in right-of-use ("ROU") assets and lease liabilities on the 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. The Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments.

Lease payments included in the measurement of the lease liability are comprised of fixed payments. Variable lease payments are excluded from the ROU asset and lease liability and are recognized in the period in which the obligation for those payments is incurred. Variable lease payments are presented in the Consolidated Statements of Operations in the same line item as expenses arising from fixed lease payments for operating leases. The Company has lease agreements that include lease and non-lease components, which the Company accounts for as a single lease component for all underlying asset categories.

The lease term for all of the Company's leases include 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 Consolidated Balance Sheets. 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.

Recent Accounting Developments - Guidance Adopted in 2020

ASU 2018-15 - In August 2018, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2018-15, Intangibles-Goodwill and Other-Internal-Use Software (Subtopic 350-40): ("ASU 2018-15"), relating to a customer's accounting for implementation, set-up, and other upfront costs incurred in a cloud computing arrangement that is hosted by a vendor. Under the new guidance, a customer will apply the same criteria for capitalizing implementation costs as it would for an arrangement that has a software license. The new guidance does not affect the accounting for the service element of a hosting arrangement that is a service contract. The new guidance also prescribes the balance sheet, income statement and cash flow classification of the capitalized software costs and related amortization expense and requires additional quantitative and qualitative disclosures. ASU 2018-15 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019 for public companies. The Company adopted this guidance on January 1, 2020. The adoption did not have a material impact on the Company's Consolidated Financial Statements or related disclosures.

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 (i) changes in unrealized gains and losses, (ii) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and (iii) 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. The Company adopted this guidance on January 1, 2020. The adoption did not have a material impact on the Company's Consolidated Financial Statements or related disclosures.

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"). ASU 2017-04 simplifies the recognition and measurement of a goodwill impairment loss by eliminating Step 2 of the quantitative goodwill impairment test. The guidance requires a one-step impairment test in which an entity compares the fair value of a reporting unit with its carrying amount and recognizes an impairment charge for the amount by which the carrying amount exceeds the reporting unit's fair value, if any. ASU 2017-04 is effective for fiscal years beginning after December 15, 2019 and should be applied on a prospective basis. The Company adopted this guidance on January 1, 2020. The adoption did not have a material impact on the Company's Consolidated Financial Statements or related disclosures.

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 adopted this guidance on January 1, 2020. The adoption did not have a material impact on the Company's Consolidated Financial Statements or related disclosures.

Recent Accounting Developments - Guidance Not Yet Adopted

ASU 2019-12 - In December 2019, the FASB issued ASU 2019-15, Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes ("ASU 2019-12"). This new guidance removes specific exceptions to the general principles in Topic 740. It eliminates the need for an organization to analyze whether the following apply in a given period: (i) exception to the incremental approach for intraperiod tax allocation; (ii) exceptions to accounting for basis differences when there are ownership changes in foreign investments; and (iii) exception in interim period income tax accounting for year-to-date losses that exceed anticipated losses. ASU 2019-12 also improves financial statement preparers' application of income tax-related guidance and simplifies the following: (i) franchise taxes that are partially based on income; (ii) transactions with a government that result in a step up in the tax basis of goodwill; (iii) separate financial statements of legal entities that are not subject to tax; and (iv) enacted changes in tax laws in interim periods. ASU 2019 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020. Early adoption is permitted for public business entities for periods for which financial statements have not yet been issued. The Company is currently assessing the impact that this standard will have on the Company's Consolidated Financial Statements upon adoption.

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

As of March 31, 2020, the Company held \$123.2 million in cash and cash equivalents and \$215.6 million of marketable securities which are reported at fair value on the Company's Consolidated Balance Sheets. Unrealized holding gains and losses are generally 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 or if an available-for-sale debt security's fair value is determined to be less than the amortized cost and the Company intends or is more than likely to sell the security before recovery and it is not considered a credit loss, such 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. If the unrealized loss of an available-for-sale debt security is determined to be a result of credit loss the Company would recognize an allowance and the corresponding credit loss would be included in earnings.

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.

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

| (in thousands) | As of March 31, 2020 | | | |
|---|----------------------|-----------------------|-----------------------|-------------------|
| | Cost | Gross Unrealized Gain | Gross Unrealized Loss | Fair Value |
| Cash and cash equivalents | \$ 123,231 | \$ — | \$ — | \$ 123,231 |
| Corporate debt securities | 107,152 | 65 | (119) | 107,098 |
| Commercial paper | 35,248 | 100 | — | 35,348 |
| Asset-backed securities | 68,812 | 25 | (42) | 68,795 |
| U.S. government agency bonds | 4,000 | — | — | 4,000 |
| Money market | 350 | — | — | 350 |
| Certificates of deposit | 51 | — | — | 51 |
| | <u>\$ 338,844</u> | <u>\$ 190</u> | <u>\$ (161)</u> | <u>\$ 338,873</u> |
| Included in cash and cash equivalents | \$ 123,231 | \$ — | \$ — | \$ 123,231 |
| Included in marketable securities | 215,613 | 190 | (161) | 215,642 |
| Total cash, cash equivalents, and marketable securities | <u>\$ 338,844</u> | <u>\$ 190</u> | <u>\$ (161)</u> | <u>\$ 338,873</u> |

| (in thousands) | As of December 31, 2019 | | | |
|---|-------------------------|-----------------------|-----------------------|-------------------|
| | Cost | Gross Unrealized Gain | Gross Unrealized Loss | Fair Value |
| Cash and cash equivalents | \$ 142,837 | \$ — | \$ — | \$ 142,837 |
| Corporate debt securities | 145,875 | 121 | (5) | 145,991 |
| Commercial paper | 73,659 | 53 | (2) | 73,710 |
| Asset-backed securities | 77,731 | 79 | — | 77,810 |
| U.S. government agency bonds | 11,999 | 2 | (10) | 11,991 |
| Money market | 350 | — | — | 350 |
| Certificates of deposit | 51 | — | — | 51 |
| | <u>\$ 452,502</u> | <u>\$ 255</u> | <u>\$ (17)</u> | <u>\$ 452,740</u> |
| Included in cash and cash equivalents | \$ 142,837 | \$ — | \$ — | \$ 142,837 |
| Included in marketable securities ⁽¹⁾ | 309,665 | 255 | (17) | 309,903 |
| Total cash, cash equivalents, and marketable securities | <u>\$ 452,502</u> | <u>\$ 255</u> | <u>\$ (17)</u> | <u>\$ 452,740</u> |

⁽¹⁾ As of December 31, 2019, \$9.5 million of marketable securities have maturity dates greater than 12 months and are available to convert into cash, if needed.

For the three months ended March 31, 2020 there were nominal realized gains. For the fiscal year ended December 31, 2019, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the marketable securities as of March 31, 2020 and December 31, 2019 reflect temporary impairments and are not a result of credit loss. Additionally, as these positions have been in a loss position for less than twelve months and the Company does not intend to sell these securities before recovery, the losses are recognized in other comprehensive gain (loss). The fair value of these marketable securities in unrealized loss positions was \$99.5 million and \$42.6 million as of March 31, 2020 and December 31, 2019, respectively.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the Consolidated Balance Sheets that sum to the total of the same such amounts shown in the Consolidated Statements of Cash Flows.

| (in thousands) | March 31, 2020 | March 31, 2019 |
|--|-------------------|------------------|
| Cash and cash equivalents | \$ 123,231 | \$ 96,349 |
| Restricted cash | 2,662 | 2,579 |
| Cash, cash equivalents, and restricted cash shown in the Consolidated Statements of Cash Flows | <u>\$ 125,893</u> | <u>\$ 98,928</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, 2020 | December 31, 2019 |
|--------------------------|------------------|-------------------|
| Raw materials | \$ 5,322 | \$ 6,544 |
| Work-in-process | 2,900 | 3,660 |
| Finished goods | 4,609 | 3,837 |
| Total inventories | <u>\$ 12,831</u> | <u>\$ 14,041</u> |

The Company recorded a reserve for inventory of \$0.2 million as of March 31, 2020 and December 31, 2019.

Note 5. Debt

The Company's debt consists of the following:

| (in thousands) | March 31, 2020 | December 31, 2019 |
|--|-------------------|-------------------|
| Senior Secured Term Loan due 2023: | | |
| Principal | \$ 150,000 | \$ 150,000 |
| Less: debt discount ⁽¹⁾ | (2,142) | (2,315) |
| Less: deferred financing ⁽¹⁾ | (289) | (311) |
| Net carrying value of the Senior Secured Term Loan | <u>\$ 147,569</u> | <u>\$ 147,374</u> |
| Convertible Notes due 2023 ⁽²⁾: | | |
| Principal | \$ 2,825 | \$ 2,825 |
| Less: debt discount ⁽¹⁾ | (625) | (659) |
| Less: deferred financing ⁽¹⁾ | (33) | (35) |
| Net carrying value of the Convertible Notes | <u>\$ 2,167</u> | <u>\$ 2,131</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.

⁽²⁾ The Convertible Notes are currently convertible as the last reported sale price of the Company's common stock was equal to or more than 130% of the conversion price for at least 20 trading days of the 30 consecutive trading days ending on the last day of the quarter.

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 unsecured Convertible Senior Notes due in 2023 ("the Convertible Notes"). Under the terms of the Exchange Agreements, the Holders agreed to exchange an aggregate principal amount of \$219.3 million of Convertible Notes held by them in exchange for an aggregate of approximately 39.0 million shares of Company common stock, par value \$0.01 per share. In addition, pursuant to the Exchange Agreements, the Company made aggregate cash payments of \$1.0 million to the Holders to satisfy accrued and unpaid interest to the closing date of the transactions, along with cash in lieu of fractional shares. These transactions resulted in \$190.4 million in additional paid-in-capital and common stock of \$0.4 million on the Consolidated Balance Sheets as of March 31, 2019. Additionally, the Company recognized a net loss on the exchange of debt of \$36.1 million on the Consolidated Statements of Operations for the three months ended March 31, 2019.

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

The following table sets forth interest expense recognized related to the Company's debt for the three months ended March 31, 2020 and 2019, respectively:

| (in thousands) | Three Months Ended March 31, | |
|------------------------------------|------------------------------|----------|
| | 2020 | 2019 |
| Contractual interest expense | \$ 3,585 | \$ 4,813 |
| Amortization of debt discount | \$ 206 | \$ 1,559 |
| Amortization of deferred financing | \$ 25 | \$ 83 |

Note 6. 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, | |
|---|------------------------------|--------|
| | 2020 | 2019 |
| Expected stock price volatility | 75.2 % | 74.2 % |
| Risk free interest rate | 1.7 % | 2.5 % |
| Expected life of options (years) ⁽¹⁾ | 5.67 | 5.68 |
| Expected annual dividend per share | \$ — | \$ — |

⁽¹⁾ The average expected life is determined using actual historical data.

A summary of the Company's stock options for the three months ended March 31, 2020 were as follows:

| | Number of Shares (in thousands) | Weighted Average Exercise Price | Weighted Average Remaining Years | Aggregate Intrinsic Value (in millions) |
|--|---------------------------------------|---------------------------------------|--|--|
| Options outstanding, December 31, 2019 | 16,724 | \$ 9.15 | | |
| Granted | 3,944 | \$ 9.57 | | |
| Exercised | (968) | \$ 6.28 | | |
| Forfeited | (355) | \$ 10.40 | | |
| Expired | (259) | \$ 13.08 | | |
| Options outstanding, March 31, 2020 | 19,086 | \$ 9.30 | 7.0 | \$ 23.5 |
| Vested and unvested expected to vest, March 31, 2020 | 17,629 | \$ 9.23 | 6.8 | \$ 23.4 |
| Exercisable at March 31, 2020 | 10,808 | \$ 8.59 | 5.4 | \$ 21.4 |

As of March 31, 2020, the total unrecognized compensation cost related to non-vested stock options granted was \$42.8 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, 2020 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, 2019 | 5,792 | \$ 11.18 | | |
| Granted | 3,600 | \$ 10.25 | | |
| Vested | (1,600) | \$ 8.94 | | |
| Forfeited | (230) | \$ 10.67 | | |
| Non-vested units as of March 31, 2020 | <u>7,562</u> | \$ 11.18 | 2.7 | \$ 69.9 |

All non-vested units are expected to vest over their normal term. As of March 31, 2020, there was \$73.4 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of three years.

Compensation Expense Related to Equity Awards

The following table summarizes information related to compensation expense recognized in the Consolidated Statements of Operations related to the equity awards:

| (in thousands) | Three Months Ended March 31, | |
|--|------------------------------|------------------|
| | 2020 | 2019 |
| Equity compensation expense recognized in: | | |
| Research and development expense | \$ 5,253 | \$ 5,032 |
| Selling, general, and administrative expense | 7,343 | 7,712 |
| Total equity compensation expense | <u>\$ 12,596</u> | <u>\$ 12,744</u> |

Note 7. 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, 2020 are identified in the following table:

| (in thousands) | Level 2 | Total |
|------------------------------|-------------------|-------------------|
| Assets: | | |
| Commercial paper | \$ 35,348 | \$ 35,348 |
| Asset-backed securities | 68,795 | 68,795 |
| Corporate debt securities | 107,098 | 107,098 |
| U.S. government agency bonds | 4,000 | 4,000 |
| Money market funds | 3,378 | 3,378 |
| | <u>\$ 218,619</u> | <u>\$ 218,619</u> |

| (in thousands) | Level 2 | Level 3 | Total |
|--------------------------------------|-----------------|------------------|------------------|
| Liabilities: | | | |
| Contingent consideration payable | \$ — | \$ 23,612 | \$ 23,612 |
| Deferred compensation plan liability | 3,055 | — | 3,055 |
| | <u>\$ 3,055</u> | <u>\$ 23,612</u> | <u>\$ 26,667</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, 2019 are identified in the following table:

| (in thousands) | Level 2 | Total |
|------------------------------|-------------------|-------------------|
| Assets: | | |
| Commercial paper | \$ 73,710 | \$ 73,710 |
| Asset-backed securities | 77,810 | 77,810 |
| Corporate debt securities | 145,991 | 145,991 |
| U.S. government agency bonds | 11,991 | 11,991 |
| Money market funds | 4,768 | 4,768 |
| | <u>\$ 314,270</u> | <u>\$ 314,270</u> |

| (in thousands) | Level 2 | Level 3 | Total |
|--------------------------------------|-----------------|------------------|------------------|
| Liabilities: | | | |
| Contingent consideration payable | \$ — | \$ 22,681 | \$ 22,681 |
| Deferred compensation plan liability | 4,419 | — | 4,419 |
| | <u>\$ 4,419</u> | <u>\$ 22,681</u> | <u>\$ 27,100</u> |

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 Convertible Notes at March 31, 2020 was \$4.7 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, 2020 or December 31, 2019.

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 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.

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. Gains and losses are included in the Consolidated Statements 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 following significant unobservable inputs were used in the valuation of the contingent consideration payable of Callidus for the ATB-200 Pompe program:

| Contingent Consideration Liability | Fair Value as of March 31, 2020 (in thousands) | Valuation Technique | Unobservable Input | Range |
|------------------------------------|---|---|--|-------------|
| Clinical and regulatory milestones | \$ 22,985 | Probability weighted discounted cash flow | Discount rate | 9.4% |
| | | | 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 following table shows the change in the balance of contingent consideration payable for the three months ended March 31, 2020 and March 31, 2019, respectively:

| (in thousands) | Three Months Ended March 31, | |
|--|------------------------------|-----------|
| | 2020 | 2019 |
| Balance, beginning of the period | \$ 22,681 | \$ 19,700 |
| Changes in fair value during the period, included in the Consolidated Statements of Operations | 931 | 1,383 |
| Adjustment for contingent consideration paid in stock | — | (316) |
| Balance, end of the period | \$ 23,612 | \$ 20,767 |

Deferred Compensation Plan - Investment and Liability

The Deferred Compensation Plan (the "Deferral Plan") provides certain key employees and members of the Board of Directors with an opportunity to defer the receipt of such participant's base salary, bonus, and director's fees, as applicable. Deferral Plan assets are classified as trading securities and recorded at fair value with changes in the investment's fair value recognized in the period they occur. The asset investments consist of market exchanged mutual funds. The Company considers its investments in marketable securities as available-for-sale and classifies these assets and related liability within the fair value hierarchy as Level 2, primarily utilizing broker quotes in a non-active market for valuation of these securities.

Note 8. 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, | |
|--|------------------------------|--------------|
| | 2020 | 2019 |
| Numerator: | | |
| Net loss attributable to common stockholders | \$ (88,948) | \$ (120,299) |
| Denominator: | | |
| Weighted average common shares outstanding — basic and diluted | 256,968,248 | 213,519,287 |

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, | |
|---|-----------------|--------|
| | 2020 | 2019 |
| Options to purchase common stock | 19,086 | 18,331 |
| Convertible notes | 462 | 5,017 |
| Outstanding warrants, convertible to common stock | 2,554 | 2,554 |
| Unvested restricted stock units | 7,562 | 6,010 |
| Total number of potentially issuable shares | 29,664 | 31,912 |

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

Overview

We are a global, patient-dedicated biotechnology company focused on discovering, developing, and delivering novel medicines for rare diseases. We have a portfolio of product opportunities led the first, oral monotherapy for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease in the clinic, and an industry leading rare disease gene therapy portfolio.

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 ("E.U."), United Kingdom ("U.K."), and Japan, with multiple additional approvals granted and applications pending in several geographies around the world.

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 ("BTD") to AT-GAA for the treatment of late onset Pompe disease.

We have established an industry leading gene therapy portfolio of potential therapies for people living with rare metabolic diseases, through a license with Nationwide Children's Hospital ("Nationwide Children's") and an expanded collaboration with the University of Pennsylvania ("Penn"). Our pipeline includes gene therapy programs in rare, neurologic lysosomal disorders ("LDs"), specifically: CLN6, CLN3, and CLN1 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD"), Niemann-Pick Type C ("NPC"), Mucopolysaccharidosis Type IIIB ("MPSIIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPSIIIA"). Our expanded collaboration with Penn also provides us with exclusive disease-specific access and the option rights to develop potentially disruptive new gene therapy platform technologies and programs for most LDs and a broader portfolio of more prevalent rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy, and select other muscular dystrophies. In the first quarter of 2020, the FDA granted Fast Track designation to the CLN3 Batten disease gene therapy, AT-GTX-502, for the treatment of pediatric patients less than 18 years of age.

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 programs in Fabry and Pompe, we are leveraging our global capabilities to develop and expand our robust pipeline in genomic medicine. We have made significant progress toward fulfilling our vision of building a leading global biotechnology company focused on rare metabolic diseases.

Our operations have not yet been significantly impacted from the novel coronavirus ("COVID-19") pandemic. We have maintained operations in all geographies, secured our global supply chain for our commercial and clinical products, and maintained our clinical trials, with minimum disruptions. Our ability to continue to operate without any significant disruptions will depend on the continued health of our employees, the ongoing demand for Galafold[®] and the continued operation of our global supply chain. We have continued to provide uninterrupted access to medicines for those in need of treatment, while prioritizing the health and safety of our global workforce. However, our results of operations in future periods may be negatively impacted by unknown future impacts from the COVID-19 pandemic.

Highlights of our progress include:

- *Commercial and regulatory success in Fabry disease.* For the three months ended March 31, 2020, Galafold[®] revenue totaled \$60.5 million, an increase of \$26.5 million compared to the same period in the prior year, with minimal impact from changes in ordering patterns related to the COVID-19 pandemic. We continue to see strong commercial momentum and expansion into additional geographies. In countries we have been operating the longest, such as Germany and the U.K., 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 completed enrollment in our global Phase 3 pivotal study of AT-GAA (ATB200-03, also known as "PROPEL") with 123 participants at 59 global sites. The U.S. FDA granted BTX for AT-GAA for the treatment of late-onset Pompe disease. Additionally, the British Medicines and Healthcare Products Regulatory Agency issued a Promising Innovative Medicine designation ("PIM") for AT-GAA for the treatment of late-onset Pompe disease. Currently, we have not experienced any significant changes to the study as a result of the COVID-19 pandemic.
- *Pipeline advancement and growth.* We have established an industry leading gene therapy portfolio of medicines for people living with rare metabolic diseases through a license with Nationwide Children's and an expanded collaboration with Penn. During the first quarter of 2020, we initiated the long-term follow-up of the initial participants in the CLN6 Phase 1/2 study. In 2019, we reported positive interim clinical results for the first eight patients in our ongoing CLN6 clinical study. Additionally, in 2019 the research collaboration with Penn was expanded to pursue research and development of novel gene therapies for Pompe disease, Fabry disease, CDD, NPC, MPSIIIB, as well as a next generation program in MPSIIIA. Amicus has rights for additional collaborative research programs in rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and select other muscular dystrophies.
- *Manufacturing.* We continue to manufacture our Pompe biologic at commercial scale (1,000L) for our pivotal PROPEL study and early commercial inventory. Our supply agreement with WuXi Biologics and current capacity are expected to produce sufficient quantities to support commercial needs after receipt of applicable regulatory approvals if obtained. For gene therapy, we are working closely with our strategic partners at Catalent Biologics and Thermo Fisher Scientific to support our clinical manufacturing capabilities for our active preclinical lysosomal disorder programs in development at Penn and the Amicus intrathecal AAV Batten disease gene therapy programs. Through the first quarter of 2020, our global supply chains have not been interrupted and we have thus far maintained our ability to manufacture Galafold® as well as our Pompe biologic, AT-GAA, during the COVID-19 pandemic.
- *Financial strength.* Total cash, cash equivalents, and marketable securities as of March 31, 2020 was \$338.9 million. The current cash position, considering the COVID-19 pandemic and including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe, and gene therapy program operations well into the second half of 2022; this assumes the current minimal impact to operations, supply chain and revenue of the COVID-19 pandemic. Potential future impact of the COVID-19 pandemic, 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 E.U. and U.K. 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 mutation (variant). The approved E.U. and U.K. labels include 367 Fabry-causing mutations, which represent up to half of all patients with Fabry disease. Approvals have also been granted in over 40 countries around the world, including the U.S., E.U., U.K., Japan, and others. We plan to continue to launch Galafold® in additional countries during 2020.

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 expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies, including Fabry disease, and other indications. In October 2019, we disclosed preliminary data from a Fabry AAV gene therapy using an Amicus-engineered transgene that demonstrated high levels of GLA activity and robust GL-3 reduction in a mouse model of Fabry disease.

Novel ERT for Pompe Disease

We are leveraging our biologics capabilities 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 of AT-GAA, ATB200-03, or PROPEL in adult patients with late onset Pompe disease in December 2018.

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 in circulation to improve the uptake of active enzyme in key disease-relevant tissues, resulting in increased clearance of accumulated substrate, glycogen.

Our strategy is to enhance the body of clinical data for AT-GAA in ongoing clinical studies. Based on regulatory feedback from both the U.S. FDA and the European Medicines Agency ("EMA"), the Phase 3 PROPEL study is expected to support approval for a broad indication, including ERT-switch and treatment-naïve patients, if the results are favorable.

In October 2019, we reported additional interim data from our clinical study ATB200-02 at the 24th International Annual Congress of the World Muscle Society. Highlights included muscle function, safety, and tolerability data in patients as well as pharmacodynamic data (muscle damage biomarker, creatine kinase, disease substrate biomarker, and urine hexose tetrasaccharide). Muscle function improved in 16 out of 18 patients at 24 months. Mean six-minute walk test ("6MWT") distance improved in both ERT-naïve and ERT-switch patients with continued benefit observed out to month 24. All five ERT-naïve patients showed increases from baseline in 6MWT distance at all time points out to month 24. To date, adverse events have been generally mild and transient. AT-GAA has resulted in a low rate of infusion-associated reactions ("IARs") following over 1,500+ infusions (28 events of IARs in eight 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.

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 expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for Pompe disease and other indications.

In April 2019, we presented initial preclinical data from our investigational adeno-associated viral ("AAV") gene therapy program for Pompe disease. This initial preclinical study in Pompe knockout mice administered a single high dose of AAV gene therapy with either unmodified wild-type hGAA ("unmodified 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 robust and consistent glycogen reduction compared to unmodified hGAA AAV gene therapy, in all key tissues assessed in a Pompe mouse model. In the central nervous system, the engineered hGAA AAV gene therapy also showed robust glycogen reduction in neuronal cells, suggesting this may be an effective way to address neuronal aspects of Pompe disease. Unmodified hGAA AAV gene therapy showed minimal glycogen reduction in neuronal cells. This preclinical study provides initial validation for combining Amicus-engineered transgenes with Penn's AAV gene therapy technologies.

Gene Therapy for Various Types of Batten Disease

Through our license with Nationwide Children's, 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 ("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 ceroid lipofuscinosis, neuronal ("CLN") 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.

We have two clinical programs in CLN6 and CLN3 Batten disease, and several preclinical programs including CLN1 and other types of Batten disease.

Our Phase 1/2 study in CLN6 Batten disease completed target enrollment, with thirteen patients receiving a single administration of adeno-associated virus serotype 9 AAV-CLN6 gene therapy. In August 2019, we reported positive interim clinical data from the first eight patients in the study. The AAV-CLN6 gene therapy demonstrated a positive impact on motor and language function. Seven out of eight patients maintained stable Hamburg Motor and Language scores or had an initial change (+1 to -1 points) followed by stabilization. In October 2019, we reported additional interim clinical data further supporting the impact of one-time intrathecal AAV gene therapy in children with CLN6 Batten disease. This interim data suggested stabilization of various components of the Hamburg Motor, Language, Seizure, and Vision scores in most patients from baseline to month 12 or 24, in particular those patients treated at a younger age, compared to the progression expected in matched untreated patients.

In the fourth quarter of 2018, we announced the initiation of a Phase 1/2 study to evaluate the safety and efficacy of a single intrathecal administration of adeno-associated virus serotype 9 AAV-CLN3 gene therapy in children with CLN3 Batten disease. In the Phase 1/2 study, a total of three patients were dosed in the low dose group, and based on the safety profile to date, the data safety monitoring board cleared us to begin enrollment in the high dose cohort of up to three additional patients. One high dose patient has been dosed, with no serious adverse events to date following a single administration of AAV-CLN3 gene therapy.

CDKL5 Deficiency Disorder

We are researching a potential first-in-class protein replacement therapy approach for CDD, as well as researching a gene therapy for CDD through our collaboration with Penn. 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.

Other Preclinical Gene Therapies

We have a number of additional gene therapies in active preclinical development, including gene therapies for NPC, MPSIIIB, as well as a next generation program in MPSIIIA. Our strategy is to develop first or best in class AAV gene therapies for these rare devastating pediatric neurological lysosomal storage diseases.

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, 2020 compared to March 31, 2019

The following table provides selected financial information for the Company:

| (in thousands) | Three Months Ended March 31, | | |
|---|------------------------------|--------------|-----------|
| | 2020 | 2019 | Change |
| Net product sales | \$ 60,525 | \$ 34,046 | \$ 26,479 |
| Cost of goods sold | 6,552 | 4,055 | 2,497 |
| Cost of goods sold as a percentage of net product sales | 10.8 % | 11.9 % | (1.1)% |
| Operating expenses: | | | |
| Research and development | 89,120 | 64,593 | 24,527 |
| Selling, general, and administrative | 40,215 | 44,303 | (4,088) |
| Changes in fair value of contingent consideration payable | 931 | 1,383 | (452) |
| Depreciation and amortization | 1,764 | 991 | 773 |
| Other income (expense): | | | |
| Interest income | 1,515 | 2,639 | (1,124) |
| Interest expense | (3,729) | (6,454) | 2,725 |
| Loss on exchange of convertible notes | — | (36,123) | 36,123 |
| Other (expense) income | (8,316) | 1,086 | (9,402) |
| Income tax expense | (361) | (168) | (193) |
| Net loss attributable to common stockholders | \$ (88,948) | \$ (120,299) | \$ 31,351 |

Net Product Sales. Net product sales increased \$26.5 million during the three months ended March 31, 2020 compared to the same period in the prior year. The increase was primarily due to continued growth in the Europe, US, and Japan markets.

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 10.8% during the three months ended March 31, 2020 compared to 11.9% 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, | |
|---|------------------------------|-----------|
| | 2020 | 2019 |
| Projects | | |
| Third party direct project expenses | | |
| Galafold® (Fabry Disease) | \$ 1,761 | \$ 4,356 |
| AT-GAA (Pompe Disease) | 34,907 | 28,286 |
| Gene therapy programs | 20,995 | 2,192 |
| Pre-clinical and other programs | 1,205 | 460 |
| Total third-party direct project expenses | 58,868 | 35,294 |
| Other project costs | | |
| Personnel costs | 22,718 | 19,634 |
| Other costs | 7,534 | 9,665 |
| Total other project costs | 30,252 | 29,299 |
| Total research and development costs | \$ 89,120 | \$ 64,593 |

The \$24.5 million increase in research and development costs was primarily due to increases in gene therapy programs driven by the pipeline growth and clinical research and manufacturing costs with the advancement and enrollment of clinical studies in the Pompe program, partially offset by a decrease in expense associated with the ongoing regulatory requirements, approval in new geographies, and pediatric and other studies to support label expansion of Galafold®. There were also increases in personnel costs associated with the advancement and enrollment of clinical studies and investments in manufacturing.

Selling, General, and Administrative Expense. Selling, general, and administrative expense decreased \$4.1 million, mainly driven by reduction in third-party professional fees and travel.

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 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 (Expense) Income. The \$9.4 million variance was primarily driven by foreign exchange losses in the remeasurement of our intercompany transactions.

Income Tax Expense. The income tax expense for the first quarter of 2020 was \$0.4 million. We are subject to income taxes in various jurisdictions. Our tax liabilities are largely dependent on the distribution of pre-tax earnings among the many jurisdiction in which we operate.

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 through stock offerings, debt issuances, Galafold® revenues, collaborations, and other financing arrangements.

Cash Flow Discussion

As of March 31, 2020, we had cash, cash equivalents, and marketable securities of \$338.9 million. We invest cash in excess of our immediate requirements in 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, 2020 was \$107.9 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2020 of \$88.9 million and the net change in operating assets and liabilities of \$45.2 million. The change in operating assets was primarily due to an increase in accounts receivable by \$8.4 million due to increased commercial sales of Galafold® and a decrease in prepaid and other current assets of \$4.0 million to support the commercial activities for Galafold®. The net cash used in operations was also impacted by a decrease in accounts payable and accrued expenses of \$35.3 million, mainly related to the payment of contract manufacturing and research costs, program expenses and personnel costs.

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 Provided by Investing Activities

Net cash provided by investing activities for the three months ended March 31, 2020 was \$93.2 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 \$106.1 million for the sale and redemption of marketable securities, partially offset by \$12.1 million for the purchase of marketable securities and \$0.8 million for the acquisition of property and equipment.

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) Provided by Financing Activities

Net cash used in financing activities for the three months ended March 31, 2020 was \$1.5 million. Net cash used in financing activities primarily reflects \$7.5 million from the purchase of vested restricted stock units, partially offset by \$6.1 million from the exercise of stock options.

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.

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, including but not limited to AT-GAA, CLN6 and CLN3;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the cost of manufacturing Pompe Enzyme Replacement Therapy ("ERT" or "ATB200") 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 a pharmacological chaperone co-administered with ERT for the treatment of Pompe disease ("AT-GAA") 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, Pompe gene therapy, Fabry gene therapy, NPC, MPSIIIB and next generation MPSIIIA, including our ability to obtain regulatory approvals and commercialize these gene therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- any changes in regulatory standards relating to the 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, including Galafold®, AT-GAA and our gene therapy candidates;
- 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, partnerships, or other similar arrangements and to obtain milestone, royalty, or other payments from any such collaborators;
- our ability to adjust to changes in the European and U.K. markets in the wake of the U.K. leaving the E.U.;
- the extent to which our business could be adversely impacted by the effects of the novel coronavirus ("COVID-19") outbreak, including due to actions by us, governments, our customers or suppliers or other third parties to control the spread of COVID-19, or by other health epidemics or pandemics;
- 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 well into the second half of 2022. 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.

Nationwide Children's - Celenex has an exclusive license agreement with Nationwide Children's. Under this license agreement, Nationwide Children's is eligible to receive development and sales-based milestones of up to \$7.8 million from us for each product.

Penn - Under our expanded collaboration agreement with Penn, Penn is eligible to receive certain milestone, royalty and discovery research 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 \$88.0 million per indication. Royalty payments are based on net sales of licensed products on a licensed product-by-licensed product and country-by-country basis. We will provide \$10.0 million each year during the five-year agreement to fund the discovery research program.

GlaxoSmithKline - 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 U.S. In addition, because we reacquired worldwide rights to migalastat, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement.

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, 2020 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, 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 \$0.7 million as of March 31, 2020. 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 March 31, 2020, we had a \$150 million Senior Secured Term Loan that bears interest at a rate equal to the 3-month LIBOR plus 7.50% per year. We do not currently hedge our variable interest rate debt. The annual average variable interest rate for our variable rate debt as of March 31, 2020 was 9.7%. A hypothetical 100 basis point increase or decrease in the average interest rate on our variable rate debt would result in a \$0.4 million change in the interest expense as of March 31, 2020.

The Financial Conduct Authority has announced the intent to phase out the use of LIBOR by the end of 2021. If LIBOR is discontinued, we may need to renegotiate the terms of the Senior Secured Term Loan in order to replace LIBOR with the new standard that is established. As a result, we may incur incremental costs in transitioning to a new standard, and interest rates on our current or future indebtedness may be adversely affected by the new standard. There is currently no definitive information regarding the future utilization of LIBOR or of any particular replacement rate. As such, the potential effect of any such event on our cost of capital cannot yet be determined, but we do not expect it to have a material impact on our consolidated financial condition, results of operations, or cash flows.

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, 2019. There have been no material changes in our financial instrument portfolio or market risk exposures since our fiscal year ended December 31, 2019.

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

The following risk factor should be considered in addition to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2019.

The novel coronavirus ("COVID-19") pandemic and efforts to reduce its spread may negatively impact our business and operations.

The COVID-19 pandemic has substantially burdened healthcare systems worldwide which may impact progression of our clinical trials. Required inspections and reviews by regulatory agencies may also be delayed due to the focus of resources on COVID-19 as well as travel and other restrictions. Significant delays in the timing of our clinical trials and in regulatory reviews could adversely affect our ability to commercialize some assets in our product pipeline. Lack of normal access by patients to the healthcare system, along with concern about the continued supply of medications, may result in changes in buying patterns throughout the supply chain, including by patients, which could increase or decrease demand for our products. Similarly, we have temporarily halted in-person interactions by our employees with healthcare providers, which may decrease demand for our products. COVID-19 could also have an adverse impact on our supply chain and distribution systems, which could impact our ability to distribute our products and the ability of third parties on which we rely to fulfill their obligations to us, and could increase our expenses. In addition, the conditions created by the pandemic may intensify other risks inherent in our business, including, among other things, risks related to drug pricing and access, intellectual property protection, product safety and efficacy concerns, product liability and other litigation, and the impact of adverse global and local economic conditions.

As a result, while the financial impact on us has not been material to date, given the rapid and evolving nature of the virus, COVID-19 could negatively affect our results of operations, financial condition, liquidity and cash flows in future periods, perhaps materially. The degree to which COVID-19 affects us will depend on developments that are highly uncertain and beyond our knowledge or control, including, but not limited to, the duration and severity of the pandemic, the actions taken to reduce its transmission, and the speed with which, and extent to which, more stable economic and operating conditions resume. Should the COVID-19 pandemic and any associated recession or depression continue for a prolonged period, our results of operations, financial condition, liquidity, and cash flows could be materially impacted by lower revenues and profitability and a lower likelihood of effectively and efficiently developing and launching new medicines.

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 | <u>First Amendment to the Amended and Restated Research, Collaboration & License Agreement dated December 20, 2019 by and between Amicus Therapeutics, Inc. and the Trustees of the University of Pennsylvania.</u> |
| ++10.2 | <u>Second Amendment to the Amended and Restated Research, Collaboration & License Agreement dated March 26, 2020 by and between Amicus Therapeutics, Inc. and the Trustees of the University of Pennsylvania.</u> |
| 31.1 | <u>Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended</u> |
| 31.2 | <u>Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended</u> |
| 32.1 | <u>Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</u> |
| 101.INS | Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document |
| 101.SCH | Inline XBRL Taxonomy Extension Schema Document |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.LAB | Inline XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Linkbase Document |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document |
| 104 | Cover Page Interactive Data File (formatted in Inline XBRL and included in Exhibit 101) |

++ Subject to confidential treatment request.

CONFIDENTIAL
Execution Version

**FIRST AMENDMENT TO AMENDED AND RESTATED
RESEARCH, COLLABORATION AND LICENSE AGREEMENT**

This FIRST AMENDMENT (the "**Amendment**"), dated December 20, 2019 (the "**Amendment Date**"), is entered into by and between The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation ("**Penn**"), and Amicus Therapeutics, Inc., a corporation organized under the laws of the state of Delaware ("**Licensee**"). Penn and Licensee may be referred to herein as a "**Party**" or, collectively, as "**Parties**".

WHEREAS, the Parties entered into a Research, Collaboration & License Agreement dated October 8, 2018, which was later amended pursuant to the Amended and Restated Research, Collaboration & License Agreement dated May 28, 2019 (the "**Collaboration Agreement**"), pursuant to which, among other things, Penn and Licensee are conducting a research program for the pre-clinical development of certain gene therapy products intended to treat certain specified indications; and

WHEREAS, the Parties now wish to amend certain provisions of the Collaboration Agreement as set forth in this Amendment.

NOW, THEREFORE, in consideration of the foregoing and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

1. The Research Plan and timeline contained in **Exhibit B** of the Agreement for the [***] programs is hereby amended to include the additional work and timeline outlined in the updated Exhibit B to this First Amendment.
2. The Research Program Budget contained in **Exhibit C** of the Agreement for the [***] is hereby amended to include the new Research Program Budget contained in Exhibit C to this First Amendment. The payment schedule contained in Exhibit C of the Agreement is amended and restated in its entirety as set forth in Exhibit C to this First Amendment.
3. This First Amendment and the Agreement contains the entire understanding between the Parties and supersedes any and all prior agreements, understandings and arrangements whether written or oral between the Parties with respect to the matters contained in the Agreement and this First Amendment. No amendments, changes, modifications or alterations of the terms and conditions of this First Amendment shall be binding upon any Party, unless in writing and signed by an authorized representative of each Party.
4. All terms and conditions of the Agreement not changed by this First Amendment shall remain in full force and effect.

5. Signatures on this First Amendment may be communicated by facsimile or e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by facsimile or e-mail, which signatures shall be deemed originals. If executed in counterparts, the Amendment shall be effective as if simultaneously executed.

[Remainder of the page intentionally left blank; signature page follows.]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the Amendment Date.

**THE TRUSTEES OF THE UNIVERSITY
OF PENNSYLVANIA**

By: /s/ John S. Swartley, PhD

Name: John S. Swartley, PhD

Title: Associate Vice Provost for

Research and Managing Director, Penn Center for Innovation

□AMICUS THERAPEUTICS, INC.

By: /s/ John F. Crowley

Name: John F. Crowley

Title: Chairman and CEO

Read and Acknowledged:

By: /s/ Dr. James M. Wilson

Name: Dr. James Wilson

Title: Director, Gene Therapy Program

Exhibit B

[***]

Exhibit C

[***]

CONFIDENTIAL
Execution Version

**SECOND AMENDMENT TO AMENDED AND
RESTATED
RESEARCH, COLLABORATION AND LICENSE AGREEMENT**

This SECOND AMENDMENT (the “**Amendment**”), dated March 26, 2020 (the “**Amendment Date**”), is entered into by and between The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation (“**Penn**”), and Amicus Therapeutics, Inc., a corporation organized under the laws of the state of Delaware (“**Licensee**”). Penn and Licensee may be referred to herein as a “**Party**” or, collectively, as “**Parties**”.

WHEREAS, the Parties entered into that certain Amended and Restated Research, Collaboration & License Agreement dated May 28, 2019, as amended through a first amendment dated December 20, 2019 (collectively, the “**Collaboration Agreement**”), pursuant to which, among other things, Penn and Licensee are conducting a research program for the pre-clinical development of certain gene therapy products intended to treat certain specified indications; and

WHEREAS, the Parties entered in to that certain Process Development Research & License Agreement dated March 26, 2020 (the “**PD Agreement**”), pursuant to which, among other things, Penn and Licensee are conducting a research program for the development and/or optimization of manufacturing technology for gene therapy products;

WHEREAS, the Parties now wish to further amend certain provisions of the Collaboration Agreement as set forth in this Amendment.

NOW, THEREFORE, in consideration of the foregoing and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

1. **Defined Terms.** Capitalized terms used in this Amendment and not defined herein shall have the meaning given to such terms in the Collaboration Agreement, unless otherwise specified herein.
2. **Joint Steering Committee.** Sections 4.1.2(j) and 4.1.2(k) of the Collaboration Agreement are hereby deleted in their entirety and replaced with the following:

“(j) endeavor to resolve any disagreement between the Parties arising at the JIPC, the JMC (as defined in the PD Agreement) established pursuant to the PD Agreement or any other subcommittee established under this Agreement or the PD Agreement;

(a) establish such additional subcommittees as it deems necessary to achieve the objectives and intent of each Research Program, the Discovery Program and/or the PD Research Program (as defined in the PD Agreement); and

(b) conduct such other activities as specifically assigned to the JSC under this Agreement or the PD Agreement.

As used in this Agreement, “**PD Agreement**” means that certain Process Development Research & License Agreement entered into by and between Penn and Licensee dated March 26, 2020.”

3. **Additional Financial Provisions.** The following is hereby added as Section 6.14 of the Collaboration Agreement:

“In the event that a Licensed Product is Covered by a Penn PD Patent Right or a Joint PD Patent Right (each, as defined in the PD Agreement) in a particular country, and such Licensed Product is not Covered by any Penn Patent Rights, Joint Patent Rights, or Licensed Know- How, the royalty and milestone payments under this Agreement shall apply to such Licensed Product as follows:

(a) Solely with respect to such Licensed Product and such country and solely for purposes of subsections (b) and (c) below, the definition of “Program Valid Claim” shall be deemed to refer to such Penn PD Patent Rights or Joint PD Patent Rights (each, as defined in the PD Agreement) instead of Penn Patent Rights A or Penn Patent Rights B;

(b) Licensee will pay Penn milestone payments in accordance with the terms of Section 6.2 upon the achievement of the corresponding milestone event by such Licensed Product; provided that such milestones have not previously been achieved by a Licensed Product for the same Indication;

(c) Licensee will pay Penn royalties in accordance with the terms of Section 6.3 on Net Sales of such Licensed Product.”

4. **Amendments.**

a. Section 1.19 of the Collaboration Agreement is hereby deleted in its entirety and replaced with the following:

“1.19 “**Discovery Patent Rights**” [***].”

b. Section 1.30 of the Collaboration Agreement is hereby deleted in its entirety and replaced with the following:

“1.30 “**DRG Technology**” means [***]”

c. Section 1.32 of the Collaboration Agreement is hereby amended by the deletion of: [***].

d. Section 1.50 of the Collaboration Agreement is hereby amended by adding the following to the end of Section 1.50:

“For clarity, Licensed Discovery Know-How does not include any DRG Technology.”

e. Section 1.93 of the Collaboration Agreement is hereby amended by adding the following to the end of Section 1.93:

“[***]”

f. Section 1.99 of the Collaboration Agreement is hereby deleted in its entirety and replaced with the following:

“1.99 **“Wilson Lab”** means Dr. James M. Wilson and all individuals who are under the direct supervision or control of Dr. James M. Wilson or his successor as Director of the Wilson Gene Therapy Program at Penn, provided that the Service Center Cores are not included in the Wilson Lab, including any personnel of the Service Center Cores. For clarity, and notwithstanding the foregoing sentence, Vector Operations is a department of the Wilson Lab and is included in the Wilson Lab, but the performance of Vector Core activities are specifically excluded from the Wilson Lab. It is understood that the activities of the Wilson Lab and/or Vector Operations in the performance of a Research Program are not Vector Core activities.”

For clarity, references to the Wilson Lab in the Collaboration Agreement, including in Sections 1.19, 1.47, 1.51, 1.57, 1.61, 1.67, 1.68, 1.69, 1.86 and 2.5.5 of the Collaboration Agreement, shall be deemed to refer to the Wilson Lab and/or Vector Operations.

g. The following sections are hereby added to Article 1 of the Collaboration Agreement:

“1.101 **“Vector Core”** means the performance of the following activities by Vector Operations for and on behalf of personnel and departments of Penn (including the Wilson Lab) and/or for other Third Parties: (a) vector production services, and (b)

characterization, release and stability testing of vectors, small scale through large scale, including for use in support of toxicology studies.”

“1.102 **“Vector Operations”** means a laboratory at Penn that reports to Dr. James M. Wilson and focuses on the development of AAV and other parvovirus vector manufacturing processes.”

“1.103 **“DRG Exclusivity Period”** means [***].”

“1.104 **“DRG Patent Rights”** means (a) [***] and (b) any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues of any of the Patent Rights in (a), including divisions, but excluding continuations-in-part except to the extent of claims solely supported in the specification and entitled to the priority date of the parent application for any of the foregoing, and (c) any corresponding foreign Patent Rights to the foregoing; provided, however, that the foregoing shall not include [***].”

“1.105 **“Exclusive DRG Technology Indications”** means (a) CDKL5 Deficiency Disorder, (b) Pompe Disease, (c) Fabry Disease, (d) NPC, (e) MPS IIIA, (f) MPS IIIB, and (g) [***], as each of the foregoing indications are defined in Sections 1.32 and 1.46.”

“1.106 **“Exploratory DRG Technology Indications”** means, [***].”

“1.107 **“Licensee Product”** means a gene therapy product, other than a Licensed Product or Designated Product, for which Licensee has rights with respect to the research, development or commercialization of the gene therapy product, itself or with or through any Third Party, for a Licensee Product Option Indication.”

“1.108 **“Licensee Product Option Indications”** means the following specific disease or condition: [***].”

h. The Collaboration Agreement is hereby amended by the addition of the following Section 2.5.5:

“2.5.5 In the event Penn desires to grant a for-profit, commercial entity (other than such an entity controlled (as such term is defined in Section 1.3) or formed by Tom Hamilton or the Cure FA Foundation) a license for the indication [***] under Patent Rights Controlled by Penn and conceived and reduced to practice by the Wilson Lab, Penn shall provide Licensee written notice and provide Licensee a right of first negotiation to negotiate with Penn terms for such license. Upon written notification from Penn (“**ROFN Notice**”), Licensee will have [***] from the date of such Offer Notice to provide written notice of exercise to Penn that Licensee desires to negotiate terms for such license with Penn (“**Exercise Notice**”). Upon Penn’s receipt of such Exercise Notice, Penn and Licensee shall negotiate in good faith for a period of up to [***] for such license agreement (“**Negotiation Period**”). For clarity, the foregoing shall not restrict Penn from negotiating terms for, or granting such license to, (a) a non-profit or non-commercial entity, or (b) any Third Party in the event (i) Licensee does not provide an Exercise Notice within such [***] period from the date of such Offer Notice, (ii) Penn and Licensee are unable to agree to the terms of such license, or (iii) Penn and Licensee are unable to enter into an agreement for such license after good faith negotiations during the Negotiation Period.”

- i. Section 2.9 of the Collaboration Agreement is hereby amended by the addition of the following sentence at the end of Section 2.9:

“[***].”

- j. The Collaboration Agreement is hereby amended by the addition of the following Section 2.13:

“2.13 **Non-Exclusive DRG Technology License Option.** During the period beginning on the Amendment Date until expiration of the Discovery Term (“**Non-Exclusive DRG Option Period**”), Penn hereby grants to Licensee an option (the “**Non-Exclusive DRG Option**”) to obtain, on a Licensee Product Option Indication-by-Licensee Product Option Indication basis, a non-exclusive, worldwide, royalty-bearing right and license, with the right to sublicense only in conjunction with a license to a Licensee Product and subject to the provisions of Section 5.6, under the DRG Patent Rights and the Know-How within the DRG Technology, to make, have made, use, sell, offer for sale, and import Licensee Products for such Licensee Product Option Indication. Licensee may exercise the Non- Exclusive DRG Option by providing written notice to Penn of exercise of such Non- Exclusive DRG Option during the Non- Exclusive DRG Option Period identifying the specific Licensee Product Option Indication subject to such exercise. Licensee shall only disclose such Licensee Product Option Indication in such exercise notice and shall not disclose to Penn additional information relating to any Licensee Product. If Licensee exercises such Non-Exclusive DRG Option, then the license grant set forth in Section 5.8.1(d) shall come into effect upon Penn’s receipt of payment of the Non-Exclusive DRG Option Fee with respect to such Licensee Product Option Indication. The option set forth in this Section 2.13 shall terminate upon early termination of the Agreement.”

k. Section 3.3.2 is hereby deleted in its entirety.

- l. Section 5.1(b) is hereby deleted in its entirety and replaced with the following:

“(b)(i) an exclusive, worldwide, royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under Penn Patent Rights B and (ii) a non-exclusive, world-wide royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under Licensed Discovery Know-How, in each case ((i) and (ii)), to make, have made, use, sell, offer for sale, and import Designated Products for the Indications in the Field of Use during the Term;”

- m. Section 5.4 of the Agreement is hereby deleted in its entirety and replaced with the following:

“**Retained Rights.** Notwithstanding the License or DRG Technology License, Penn retains the right under the Penn Patent Rights and the DRG Patent Rights to (a) conduct educational, research and clinical / patient care activities itself (including sponsored research) and (b) authorize non- commercial third parties to conduct education, non- commercial research and clinical / patient activities, in each case of subpart (a) and (b) related to the Indications in the Field of Use; provided however that Penn shall not have the right to use or authorize the use of any Licensed Product for an Indication that is under active clinical testing or being sold by Licensee in the conduct of clinical or patient care activities without Licensee’s consent.”

- n. Section 5.5 of the Agreement is hereby deleted in its entirety and replaced with the following:

“**U.S. Government Rights.** The License and the DRG Technology License are expressly subject to all applicable provisions of any license to the United States Government executed by Penn and is subject to any overriding obligations to the United States Federal Government under 35 U.S.C. §§200- 212, applicable governmental implementing regulations, and the U.S. Government sponsored research agreement or other guidelines, including that products that result from intellectual property funded by the United States Federal Government that are sold in the United States be substantially manufactured in the United States. If so requested by Licensee, Penn shall reasonably cooperate with and assist Licensee to seek and obtain a waiver from the appropriate Regulatory Authorities with respect to such manufacturing requirement.”

- o. The Collaboration Agreement is hereby amended by the addition of the following Section 5.8:

“5.8 **DRG Technology License.**

i. **Grant of DRG Technology License.** Subject to the terms and conditions of this Agreement, Penn hereby grants to Licensee (the below rights, the “**DRG Technology License**”):

(a) (i) an exclusive, worldwide, royalty-bearing (solely to the extent provided in Section 6.3) right and license, with the right to sublicense only in conjunction with a Designated Product for such Exclusive DRG Technology Indication subject to the provisions Section 5.6, under the DRG Patent Rights and (ii) a non-exclusive, worldwide, royalty-bearing right and license, with the right to sublicense only in conjunction with a Designated Product subject to the provisions Section 5.6 under the DRG Technology, in each case ((i) and (ii)), to make, have made, use, sell, offer for sale, and import Designated Products for the Exclusive DRG Technology Indications in the Field of Use for the Exclusivity Period. Following the expiration of the Exclusivity Period, the DRG Technology License granted under this Section 5.8.1(a)(i) shall convert to a non- exclusive, worldwide, royalty-bearing right and license with the right to sublicense only in conjunction with a Designated Product subject to the provisions Section 5.6;

(b) a non-exclusive, worldwide, royalty-bearing (solely to the extent provided in Section 6.3) right and license, with the right to sublicense only in conjunction with a Designated Product for such Exploratory DRG Technology Indication subject to the provisions of Section 5.6, under DRG Patent Rights and the DRG Technology to make, have made, use, sell, offer for sale, and import Designated Products for the Exploratory Indications;

(c) a non-exclusive, worldwide, royalty-bearing (solely to the extent provided in Section 6.3) right and license, with the right to sublicense only in conjunction with a Licensed Product for such Exclusive DRG Technology Indication subject to the provisions of Section 5.6, under DRG Patent Rights and the DRG Technology to make, have made, use, sell, offer for sale, and import Licensed Products for the Exclusive DRG Technology Indications; and

(d) on a Licensee Product Option Indication-by-Licensee Product Option Indication basis and subject to Licensee's exercise of the Non-Exclusive DRG Option and payment of the Non-Exclusive DRG Option Fee, a non-exclusive, worldwide, royalty- bearing right and license, with the right to sublicense only in conjunction with a Licensee Product, under the DRG Patent Rights and DRG Technology to make, have made, use, sell, offer for sale, and import Licensee Products for the Licensee Product Option Indication.

ii. **Limited Exclusivity.** The exclusive license grant set forth in Section 5.8.1(a) is subject to any applicable requirement, order, specific written request or decree by a Regulatory Authority to Penn to make such DRG Technology available to a Third Party on a non-exclusive basis for a specified Exclusive DRG Technology Indication. In such event, Penn shall provide written notice to Licensee of the foregoing, and any exclusive license grant set forth in Section 5.8.1(a) during the DRG Exclusivity Period shall convert to a non-exclusive license grant with respect to such Exclusive DRG Technology Indication to the extent necessary to accommodate Penn's compliance with such requirement, order, specific written request or decree.

iii. **Diligence.** In the event an IND is filed for a Designated Product for an Exclusive DRG Technology Indication and such Designated Product does not utilize the

DRG Technology, upon written notification by Penn, the exclusive license grant under Section 5.8.1(a) with respect to such Exclusive DRG Technology Indication shall convert to a non-exclusive, worldwide, royalty-bearing right and license without the right to sublicense (except in conjunction with a sublicense to a Licensed Product subject to the provisions Section 5.6).

iv. **Indication Restriction.** Subject to Section 5.8.3 and Penn's retained rights set forth in Section 5.4 and Section 5.8.2, during the DRG Exclusivity Period, Penn shall not license to any Third Party the DRG Patent Rights for a product for an Exclusive DRG Technology Indication.

e. The Collaboration Agreement is hereby amended by the addition of the following Section 6.15:

"6.15 Financials Related to the DRG Technology License.

i. Financials Related to License Granted Pursuant to Section 5.8.1(d)

1. **Upfront Payment.** On a Licensee Product Option Indication-by-Licensee Product Option Indication basis, within [***] of Licensee's exercise of the Non-Exclusive DRG Option with respect to such Licensee Product Option Indication, Licensee shall pay Penn a non-refundable and non-creditable license issue fee of: (a) [***] if Licensee exercises such Non-Exclusive DRG Option for such Licensee Product Option Indication prior to the first dosing of the first patient in a Clinical Study of a Licensee Product for such Licensee Product Option Indication or (b) [***] if Licensee exercises such Non-Exclusive DRG Option for such Licensee Product Option Indication after the first dosing of the first patient in a Clinical Study of a Licensee Product for such Licensee Product Option Indication.

2. **Milestones.** In addition, and as additional consideration for the DRG Technology License set forth in Section 5.8.1(d), Licensee will pay Penn the following milestone payments upon the achievement of the first Licensee Product to achieve the corresponding milestone for each Licensee Product Option Indication, whether achieved by Licensee or an Affiliate or Sublicensee. Licensee shall promptly notify Penn in writing of the achievement of any such milestone and Licensee shall pay Penn in full the corresponding milestone payment within [***] of such achievement. For clarity, each milestone payment set forth below is non-refundable, non-creditable and is not an advance against Royalties due to Penn or any other amounts due to Penn.

| Milestone (payable once per Licensee Product Option Indication) | Milestone Payment (in U.S. dollars) |
|--|--|
| [***] | [\$[***]] |

| Milestone (payable once per Licensee Product Option Indication) | Milestone Payment (in U.S. dollars) |
|--|--|
| [***] | \$[***] |
| [***] | \$[***] |
| Total Milestone Payments per Licensee Product Option Indication | \$[***] |

3. **Royalty.** In addition, and as further consideration for the DRG Technology License set forth in Section 5.8.1(d), on a Licensee Product-by-Licensee Product basis for each Licensee Product Option Indication and during the applicable Licensee Product Royalty Period, Licensee shall pay to Penn a non-refundable, non-creditable royalty of [***] of worldwide Net Sales of Licensee Product for such Licensee Product Option Indication. Licensee’s obligation to pay Penn such royalty will continue on a country-by-country and Licensee Product-by- Licensee Product basis from the date of First Commercial Sale of such Licensee Product for such Licensee Product Option Indication in a country until the latest of (a) the expiration or abandonment of the last Valid Claim within the DRG Patent Rights covering such Licensee Product in such country, (b) [***] after First Commercial Sale of such Licensee Product in such country, (c) the expiration of the Regulatory Exclusivity with respect to such Licensee Product; [***] (such royalty period, the “**Licensee Product Royalty Period**”). In addition, the royalty under this Section 6.15.1.3 shall be subject to the royalty reductions set forth in Section 6.3.3, applied *mutatis mutandis*. For the avoidance of doubt, no additional royalty shall be due under this Section 6.15 with respect to Net Sales of any Licensed Product, including any Designated Product. For the avoidance of doubt, such products are subject to royalty obligations solely pursuant to Section 6.3.

4. As used in this Section 6.15.1, (a) “Net Sales” shall have the meaning ascribed to such term as set forth in Section 1.59, substituting Licensee Product for each reference to Licensed Product in such section and (b) “Valid Claim” shall have the meaning ascribed to such term as set forth

in Section 1.98, substituting DRG Patent Rights for each reference to Penn Patent Rights in such section.”

f. The Collaboration Agreement is hereby amended by the addition of the following Section 8.7:

“8.7 DRG Patent Filing Prosecution and Maintenance, Patent Costs, Infringement.

i. Patent Filing Prosecution and Maintenance.

(a) Penn will use diligent efforts to file, and thereafter prosecute in good faith and maintain DRG Patent Right(s). DRG Patent Right(s) will be held in the name of Penn and obtained with Patent Counsel. Penn shall control all actions and decisions with respect to the filing, prosecution and maintenance of DRG Patent Right(s). For the purposes of this Agreement, “maintenance” of the DRG Patent Right(s) includes *inter partes* patent review proceedings before the USPTO or a similar patent administration outside the US. For DRG Patent Rights, Penn will instruct Patent Counsel to provide Licensee copies of patent applications when filed, notices of allowance when received, office actions when issued and office action responses when filed.

(b) In the event that DRG Technology is conceived and reduced to practice by the Wilson Lab and/or Vector Operations during the Discovery Term without the use of any Licensee funding (“**DRG Technology Improvement**”) and such DRG Technology Improvement is also incorporated into any clinical candidate of a Third Party with whom the Wilson Lab is collaborating, then Penn may file and prosecute a Patent Right that covers such clinical candidate of a Third Party with whom the Wilson Lab is collaborating incorporating such DRG Technology Improvement (“**Third Party Candidate Patent Right**”); provided, however, that if Penn files a Third Party Candidate Patent Right, Penn shall also separately file and prosecute a patent application specific for such DRG Technology Improvement distinct from such Third Party Candidate Patent Right.

ii. Patent Costs.

1. Subject to Section 8.7.2.3, within [***] after the Amendment Date, Licensee will reimburse Penn for all documented out-of-pocket costs for the filing, prosecution and maintenance of DRG Patent Right(s) for the filing, prosecution and maintenance of DRG Patent Rights, including all accrued and documented attorney fees, expenses, official and filing fees (“**DRG Patent Costs**”), incurred prior to the Amendment Date, which have not otherwise been reimbursed by Licensee or other licensees of such DRG Patent Rights (“**Historic DRG Patent Costs**”). Notwithstanding the first sentence of this Section 8.7.2.1, for DRG Patent Right(s) licensed by Penn to more than one licensee, Licensee shall be responsible for payment to Penn of a pro rata

share of such documented Historic DRG Patent Costs based on the number of licensees for such Patent Rights.

2. Licensee will bear (a) all DRG Patent Costs incurred during the Term for DRG Patent Right(s) (“**Ongoing DRG Patent Costs**”). Notwithstanding the foregoing, for DRG Patent Rights licensed by Penn to more than one licensee, Licensee shall be responsible for payment to Penn of a pro rata share of such documented Ongoing DRG Patent Costs based on the number of licensees for such DRG Patent Rights. No later than sixty (60) days prior to the end of each Calendar Year during the Term, Penn shall provide to Licensee, a good faith estimate and budget for the Ongoing DRG Patent Costs anticipated to be incurred for the next Calendar Year and, to the extent applicable, Licensee’s proportionate share of such Ongoing DRG Patent Costs. This Section 8.7.2 is subject to Section 8.7.1 above.

3. With respect to DRG Patent Right(s), Licensee shall be subject to Advance Payment. Notwithstanding whether Licensee makes an Advance Payment for any patent action, Licensee shall bear its pro rata share (based on the number of licensees for such DRG Patent Rights) of all DRG Patent Costs with respect to DRG Patent Right(s) as set forth in this Section 8.7.2, above, and shall pay such amounts within [***] of receipt of invoice for such patent actions.

4. Licensee shall also have the right, on a DRG Patent Right-by-DRG Patent Right and country-by-country basis, to (i) elect not to fund at the time of disclosure, or (ii) elect not to continue to fund, in each case (i) and (ii), its pro rata share (as determined pursuant to this Section 8.7 above) of the DRG Patent Costs with respect to any DRG Patent Right(s) in a particular country, which election may be made by Licensee upon sixty (60) days prior written notice to Penn (“**DRG Election Notice**”). If Licensee delivers a DRG Election Notice to Penn, following the expiration of such sixty (60) day period, Licensee shall have no further obligation to pay Ongoing DRG Patent Costs with respect to any DRG Patent Right identified in such DRG Election Notice in any country identified in such DRG Election Notice and any such Patent Right in any such country shall thereafter be excluded from the DRG Patent Rights.

iii. **Infringement.**

1. If either Party believes that an infringement by a Third Party with respect to any DRG Patent Right is occurring or may potentially occur, the knowledgeable Party will provide the other Party with (a) written notice of such infringement or potential infringement and (b) evidence of such infringement or potential infringement. With respect to DRG Patent Rights, Penn shall have the exclusive right to enforce such Patent Rights and institute suit for Patent Infringement. If Penn institutes such suit, then Licensee may not join such suit without the prior written consent of Penn.

2. Any recovery or settlement received in connection with any suit will be the exclusive property of Penn.

3. Licensee will reasonably cooperate and assist Penn in litigation proceedings instituted hereunder at Penn's request and expense.

4. Penn shall keep Licensee reasonably informed of the initiation and status of any action to enforce any DRG Patent Rights to which Licensee has rights hereunder.

5. If, within [***] following the date of a notice delivered pursuant to Section 8.7.3.1, infringing activity of potential commercial significance with respect to the DRG Patent Rights for a Licensee Option Indication has not been abated or Penn has not brought suit against the infringer, then payments due to Penn with respect to the DRG Patent Rights for a Licensee Product for a Licensee Product Option Indication pursuant to Section 6.15 and/or Section 8.7.2 shall be suspended for so long as the infringing activity of potential commercial significance continues unabated by a Third Party competitor of a Licensee Product for the Licensee Product Option Indication and Penn does not bring suit against the infringer relating to such Licensee Product for the Licensee Product Option Indication that is the subject of the notice of infringement under Section 8.7.3.1. If such infringement is subsequently abated, whether as a result of acts of Penn or a Third Party, Licensee's payment obligations with respect to the DRG Patent Rights for such infringed Licensee Product Option Indication shall be reinstated; provided that Licensee shall have no obligation to pay any amounts that would have been due pursuant to Section 6.15 and/or Section 8.7.2 during the period of any unabated infringement but for this Section 8.7.3.5."

c. Section 10.2.3 is hereby amended and restated in its entirety as follows:

"Other than licenses granted under the Excluded Penn IP, rights granted to Patent Rights set forth in Exhibit X or the arrangements with Third Parties described in Schedule 3.2.4 or Schedule 10.2.3, to Penn's knowledge, Penn has not entered into any arrangement with any Third Party for any Indication, Potential Indication or Exploratory Indication prior to the New Effective Date which is still in effect and pursuant to which a Third Party has license rights, or has an option to obtain rights, to any Patent Rights conceived or reduced to practice in the Wilson Lab."

d. Exhibit G is hereby amended and restated in its entirety with Exhibit G attached hereto.

e. Exhibit H is hereby amended and restated in its entirety with Exhibit H attached hereto.

f. Schedule 3.2.4 is hereby amended and restated in its entirety with Schedule 3.2.4 attached hereto.

5. **No Other Modification.** Except as specifically set forth in this Amendment, the terms and conditions of the Collaboration Agreement shall continue in full force and effect and shall apply to this Amendment. This Amendment constitutes the complete and exclusive statement of agreement between the Parties with respect to the subject matter of this Amendment, and supersedes all prior agreements and understandings, and all prior and contemporaneous (oral or written) proposals, understanding, representations, conditions, warranties, covenants and all other communications between the Parties respecting the subject matter hereof. No wa

iver, modification or amendment of any provision of this Amendment shall be valid or effective

unless made in a writing referencing this Amendment and signed by a duly authorized officer of each Party.

6. **Miscellaneous.** This Amendment may be executed in counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or a portable document format (PDF) copy of this Amendment, including the signature pages, will be deemed an original. This Amendment shall be governed by and interpreted in accordance with the laws of the Commonwealth of Pennsylvania, excluding application of any conflict of laws principles that would require application of the law of a jurisdiction outside of the Commonwealth of Pennsylvania.

[Remainder of the page intentionally left blank; signature page follows.]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the Amendment Date.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ John S. Swartley, PhD

Name: John S. Swartley, PhD

Title: Associate Vice Provost for Research and Managing
Director, Penn Center
for Innovation

AMICUS THERAPEUTICS, INC.

By: /s/ John Crowley

Name: John Crowley

Title: Chairman and CEO

Read and Acknowledged:

By: /s/ Dr. James M. Wilson

Name: Dr. James M. Wilson

Title: Director, Gene Therapy Program

Amended and Restated Exhibit G

[***]

Amended and Restated Exhibit H

[***]

Amended and Restated Schedule 3.2.4

[***]

Schedule 10.2.3

[***]

Exhibit X

[*]**

Exhibit Y

[*]**

**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 7, 2020

/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 7, 2020

/s/ Daphne Quimi

Daphne Quimi
Chief Financial Officer

