

**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, 2021

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)

3675 Market Street, Philadelphia, PA
(Address of Principal Executive Offices)

71-0869350

(I.R.S. Employer
Identification Number)

19104
(Zip Code)

(215) 921-7600

(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, par value \$0.01 per share	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	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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 26, 2021 was 266,060,455 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended March 31, 2021

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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 scope, progress, results and costs of our 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" or "cipaglucosidase alfa") and gene therapies;
- the future results of on-going preclinical research and subsequent clinical trials for cyclin-dependent kinase-like 5 ("CDKL5") deficiency disorder, Pompe gene therapy, Fabry gene therapy, Mucopolysaccharidosis Type IIIB ("MPS IIIB"), next generation Mucopolysaccharidosis Type IIIA ("MPS IIIA") and other pipeline candidates we may identify from time to time, including our ability to obtain regulatory approvals and commercialize these therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates, including AT-GAA;
- 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[®] (also referred to as "migalastat HCl") and, if our regulatory filings are accepted and approved, AT-GAA;
- 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[®] and, if our regulatory filings are accepted and approved, AT-GAA;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold[®];
- our ability to obtain market acceptance of Galafold[®] and, if our regulatory filings are accepted and approved, AT-GAA;
- 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, 2020, 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, 2020 (including the documents incorporated by reference therein) 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, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	\$ 184,833	\$ 163,240
Investments in marketable securities	232,596	320,029
Accounts receivable	44,931	46,923
Inventories	18,801	19,556
Prepaid expenses and other current assets	21,730	29,721
Total current assets	502,891	579,469
Operating lease right-of-use assets, less accumulated amortization of \$7,499 and \$7,574 at March 31, 2021 and December 31, 2020, respectively	22,363	23,296
Property and equipment, less accumulated depreciation of \$15,961 and \$14,487 at March 31, 2021 and December 31, 2020, respectively	43,445	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	20,538	19,095
Total Assets	\$ 810,034	\$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 16,110	\$ 17,063
Accrued expenses and other current liabilities	57,178	96,841
Contingent consideration payable	19,600	8,900
Operating lease liabilities	6,764	6,872
Total current liabilities	99,652	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	389,789	389,254
Contingent consideration payable	6,696	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	44,431	45,604
Other non-current liabilities	6,268	6,379
Total liabilities	559,138	600,140
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 266,007,718 and 262,063,461 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	2,680	2,650
Additional paid-in capital	2,350,507	2,308,578
Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	9,020	8,412
Unrealized loss on available-for-sale securities	(185)	(185)
Warrants	—	12,387
Accumulated deficit	(2,111,126)	(2,045,462)
Total stockholders' equity	250,896	286,380
Total Liabilities and Stockholders' Equity	\$ 810,034	\$ 886,520

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,	
	2021	2020
Net product sales	\$ 66,402	\$ 60,525
Cost of goods sold	6,539	6,552
Gross profit	59,863	53,973
Operating expenses:		
Research and development	64,117	89,120
Selling, general, and administrative	46,726	40,215
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expenses	112,918	132,030
Loss from operations	(53,055)	(78,057)
Other (expense) income:		
Interest income	165	1,515
Interest expense	(7,992)	(3,729)
Other expense	(3,200)	(8,316)
Loss before income tax	(64,082)	(88,587)
Income tax expense	(1,582)	(361)
Net loss attributable to common stockholders	\$ (65,664)	\$ (88,948)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.25)	\$ (0.35)
Weighted-average common shares outstanding — basic and diluted	264,369,317	256,968,248

See accompanying Notes to Consolidated Financial Statements

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

	Three Months Ended March 31,	
	2021	2020
Net loss	\$ (65,664)	\$ (88,948)
Other comprehensive gain (loss):		
Foreign currency translation adjustment gain, net of tax impact of \$132 and \$1,536, respectively	608	4,196
Unrealized loss on available-for-sale securities, net of tax impact of \$0 and \$(56), respectively	—	(209)
Other comprehensive income	\$ 608	\$ 3,987
Comprehensive loss	\$ (65,056)	\$ (84,961)

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, 2021

	Common Stock		Additional Paid-In Capital	Warrants	Other Comprehensive Gain (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2020	262,063,461	\$ 2,650	\$ 2,308,578	\$ 12,387	\$ 8,227	\$ (2,045,462)	\$ 286,380
Stock issued from exercise of stock options, net	488,111	4	4,157	—	—	—	4,161
Restricted stock tax vesting	897,063	—	(14,194)	—	—	—	(14,194)
Stock-based compensation	—	—	20,354	—	—	—	20,354
Warrants exercised	2,554,999	26	31,591	(12,387)	—	—	19,230
Equity component of the convertible notes	4,084	—	21	—	—	—	21
Foreign currency translation adjustment	—	—	—	—	608	—	608
Net loss	—	—	—	—	—	(65,664)	(65,664)
Balance at March 31, 2021	266,007,718	\$ 2,680	\$ 2,350,507	\$ —	\$ 8,835	\$ (2,111,126)	\$ 250,896

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

See accompanying Notes to Consolidated Financial Statements

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

	Three Months Ended March 31,	
	2021	2020
Operating activities		
Net loss	\$ (65,664)	\$ (88,948)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of debt discount and deferred financing	555	231
Depreciation and amortization	1,604	1,764
Stock-based compensation	20,354	12,596
Non-cash changes in the fair value of contingent consideration payable	471	931
Foreign currency remeasurement loss	2,846	10,662
Changes in operating assets and liabilities:		
Accounts receivable	839	(8,358)
Inventories	741	(251)
Prepaid expenses and other current assets	7,669	4,016
Accounts payable and accrued expenses	(40,191)	(35,251)
Other non-current assets and liabilities	(1,578)	(5,334)
Net cash used in operating activities	\$ (72,354)	\$ (107,942)
Investing activities		
Sale and redemption of marketable securities	163,680	106,140
Purchases of marketable securities	(76,247)	(12,088)
Capital expenditures	(868)	(806)
Net cash provided by investing activities	\$ 86,565	\$ 93,246
Financing activities		
Payment of finance leases	(368)	(21)
Purchase of vested restricted stock units	(14,194)	(7,543)
Proceeds from exercise of stock options	4,161	6,077
Proceeds from exercise of warrants	19,230	—
Net cash provided by (used in) financing activities	\$ 8,829	\$ (1,487)
Effect of exchange rate changes on cash, cash equivalents, and restricted cash	\$ (1,153)	\$ (4,265)
Net increase(decrease) in cash, cash equivalents, and restricted cash at the end of the period	21,887	(20,448)
Cash, cash equivalents, and restricted cash at beginning of period	166,162	146,341
Cash, cash equivalents, and restricted cash at the end of period	\$ 188,049	\$ 125,893
Supplemental disclosures of cash flow information		
Tenant improvements paid through lease incentives	\$ —	\$ 254
Cash paid during the period for interest	\$ 7,513	\$ 3,693
Capital expenditures unpaid at the end of period	\$ 188	\$ 589
Cash paid for taxes	\$ 2,472	\$ 1,627

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, 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, or cipa[®]glucosidase alfa/miglustat), 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. The Company initiated the rolling Biologic License Application ("BLA") submission to the FDA in the fourth quarter of 2020.

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 a research 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"), Mucopolysaccharidosis Type IIIB ("MPS IIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPS IIIA"). This research 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. In September 2020 and February 2021, the European Medicines Agency granted Priority Medicines designation and the FDA granted Fast Track Designation, respectively, to the CLN6 Batten disease gene therapy, AT-GTX-501, for the treatment of patients with variant late infantile neuronal ceroid lipofuscinosis 6 ("vLINCL6").

The Company's operations have not been significantly impacted from the novel coronavirus ("COVID-19") pandemic thus far. However, the Company continued to observe increased lag times between patient identification and Galafold[®] initiation due to the resurgence of COVID-19 into 2021. The Company has maintained operations in all geographies, secured its global supply chain for its commercial and clinical products, and maintained the operational integrity of 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 \$2.1 billion as of March 31, 2021 and anticipates incurring losses through the fiscal year ending December 31, 2021 and beyond. The Company has historically funded its operations through stock offerings, debt issuances, Galafold[®] revenues, collaborations, and other financing arrangements.

Based on current operating models, the Company believes that the current cash position, which includes expected revenues, is sufficient to fund the Company's operations and ongoing research programs to achieve self-sustainability. Potential impacts of the COVID-19 pandemic, 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 the 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, 2020. 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, 2020.

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, 2021 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 other current assets and other 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, 2021 have arisen from product sales primarily in Europe and the U.S. The Company will periodically assess the financial strength of its customers to establish allowances for anticipated losses, if any. For accounts receivable that have arisen from named patient sales, the payment terms are predetermined, and the Company evaluates the creditworthiness of each customer on a regular basis. As of March 31, 2021, 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 revenue from the sale of Galafold[®] is 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,	
	2021	2020
U.S.	\$ 20,853	\$ 17,772
Ex-U.S.	45,549	42,753
Total net product sales	<u>\$ 66,402</u>	<u>\$ 60,525</u>

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

Leases with an initial term of 12 months or less are not recorded on the 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 2021

ASU 2019-12 - In December 2019, the Financial Accounting Standard Board issued Accounting Standard Update ("ASU") 2019-12, 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 applies 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 2019-12 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020. The Company adopted this guidance prospectively on January 1, 2021. 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

The Company has evaluated recent accounting pronouncements and believes that none of them will have a material effect on the Company's Consolidated Financial Statements or related disclosures.

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

As of March 31, 2021, the Company held \$184.8 million in cash and cash equivalents and \$232.6 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, 2021			
	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	\$ 184,833	\$ —	\$ —	\$ 184,833
Corporate debt securities	20,936	—	(4)	20,932
Commercial paper	151,300	10	(3)	151,307
Asset-backed securities	13,718	1	(3)	13,716
U.S. government agency bonds	46,228	12	—	46,240
Money market	350	—	—	350
Certificates of deposit	51	—	—	51
	<u>\$ 417,416</u>	<u>\$ 23</u>	<u>\$ (10)</u>	<u>\$ 417,429</u>
Included in cash and cash equivalents	\$ 184,833	\$ —	\$ —	\$ 184,833
Included in marketable securities	232,583	23	(10)	232,596
Total cash, cash equivalents, and marketable securities	<u>\$ 417,416</u>	<u>\$ 23</u>	<u>\$ (10)</u>	<u>\$ 417,429</u>

(in thousands)	As of December 31, 2020			
	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	\$ 163,240	\$ —	\$ —	\$ 163,240
Corporate debt securities	39,525	4	(16)	39,513
Commercial paper	217,087	14	(6)	217,095
Asset-backed securities	9,420	18	—	9,438
U.S. government agency bonds	53,583	3	(4)	53,582
Money market	350	—	—	350
Certificates of deposit	51	—	—	51
	<u>\$ 483,256</u>	<u>\$ 39</u>	<u>\$ (26)</u>	<u>\$ 483,269</u>
Included in cash and cash equivalents	\$ 163,240	\$ —	\$ —	\$ 163,240
Included in marketable securities ⁽¹⁾	320,016	39	(26)	320,029
Total cash, cash equivalents, and marketable securities	<u>\$ 483,256</u>	<u>\$ 39</u>	<u>\$ (26)</u>	<u>\$ 483,269</u>

For the three months ended March 31, 2021 there were no realized gains or losses. For the fiscal year ended December 31, 2020, there were nominal realized gains. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the marketable securities as of March 31, 2021 and December 31, 2020 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 \$114.5 million and \$124.9 million as of March 31, 2021 and December 31, 2020, 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, 2021	March 31, 2020
Cash and cash equivalents	\$ 184,833	\$ 123,231
Restricted cash	3,216	2,662
Cash, cash equivalents, and restricted cash shown in the Consolidated Statements of Cash Flows	<u>\$ 188,049</u>	<u>\$ 125,893</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, 2021	December 31, 2020
Raw materials	\$ 3,709	\$ 5,547
Work-in-process	10,023	7,693
Finished goods	5,069	6,316
Total inventories	<u>\$ 18,801</u>	<u>\$ 19,556</u>

The Company recorded a reserve for inventory of \$0.2 million and \$0.1 million as of March 31, 2021 and December 31, 2020, respectively.

Note 5. Debt

The Company's debt consists of the following:

(in thousands)	March 31, 2021	December 31, 2020
Senior Secured Term Loan due 2026:		
Principal	\$ 400,000	\$ 400,000
Less: debt discount ⁽¹⁾	(7,139)	(7,434)
Less: deferred financing ⁽¹⁾	(5,371)	(5,592)
Net carrying value of the Senior Secured Term Loan	<u>\$ 387,490</u>	<u>\$ 386,974</u>
Convertible Notes ⁽²⁾:		
Principal ⁽³⁾	\$ 2,800	\$ 2,825
Less: debt discount ⁽¹⁾	(476)	(518)
Less: deferred financing ⁽¹⁾	(25)	(27)
Net carrying value of the Convertible Notes	<u>\$ 2,299</u>	<u>\$ 2,280</u>
Net carrying value of Long-term debt	<u>\$ 389,789</u>	<u>\$ 389,254</u>

⁽¹⁾ Included in the Consolidated Balance Sheets within long-term debt 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.

⁽³⁾ In the first quarter of 2021, the Company exchanged an aggregate principal amount of \$25.0 thousand of Convertible Notes in exchange for an aggregate of approximately 4.1 thousand shares of Company common stock, par value \$0.01 per share.

Interest Expense

The following table sets forth interest expense recognized related to the Company's debt for the three months ended March 31, 2021 and 2020, respectively:

(in thousands)	Three Months Ended March 31,	
	2021	2020
Contractual interest expense	\$ 7,521	\$ 3,585
Amortization of debt discount	\$ 332	\$ 206
Amortization of deferred financing	\$ 223	\$ 25

Note 6. Stockholders' Equity

In February 2021, 1,050,000 warrants were exercised at \$7.06 per share of common stock resulting in gross cash proceeds of \$7.4 million. Additionally, in March 2021, 1,294,999 warrants were exercised at \$7.98 per share of common stock and 210,000 warrants were exercised at \$7.06 per share of common stock resulting in gross cash proceeds of \$11.8 million.

Note 7. Share-Based Compensation

The Company's Amended and Restated 2007 Equity Incentive 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 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,	
	2021	2020
Expected stock price volatility	66.7 %	75.2 %
Risk free interest rate	0.4 %	1.7 %
Expected life of options (years)	5.40	5.67
Expected annual dividend per share	\$ —	\$ —

A summary of the Company's stock options for the three months ended March 31, 2021 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, 2020	14,032	\$ 9.54		
Granted	1,818	\$ 21.46		
Exercised	(493)	\$ 8.54		
Forfeited	(273)	\$ 12.40		
Options outstanding, March 31, 2021	15,084	\$ 10.96	6.7	\$ 18.5
Vested and unvested expected to vest, March 31, 2021	13,643	\$ 10.71	6.4	\$ 18.3
Exercisable at March 31, 2021	8,962	\$ 9.04	5.2	\$ 17.7

During the three months ended March 31, 2021, nominal stock options expired at a weighted average exercise price of \$15.36. As of March 31, 2021, the total unrecognized compensation cost related to non-vested stock options granted was \$38.7 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, 2021 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, 2020	7,080	\$ 11.35		
Granted	2,131	\$ 20.22		
Vested	(1,435)	\$ 17.38		
Forfeited	(310)	\$ 13.36		
Non-vested units as of March 31, 2021	<u>7,466</u>	\$ 14.14	2.7	\$ 73.8

All non-vested units are expected to vest over their normal term. As of March 31, 2021, there was \$64.5 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,	
	2021	2020
Equity compensation expense recognized in:		
Research and development expense	\$ 6,305	\$ 5,253
Selling, general, and administrative expense	14,049	7,343
Total equity compensation expense	<u>\$ 20,354</u>	<u>\$ 12,596</u>

Note 8. Assets and Liabilities Measured at Fair Value

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

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

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

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

A summary of the fair value of the Company's recurring assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of March 31, 2021 are identified in the following tables:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	\$ 151,307	\$ 151,307
Asset-backed securities	13,716	13,716
Corporate debt securities	20,932	20,932
U.S. government agency bonds	46,240	46,240
Money market funds	4,296	4,296
	<u>\$ 236,491</u>	<u>\$ 236,491</u>

(in thousands)	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	\$ —	\$ 26,296	\$ 26,296
Deferred compensation plan liability	3,946	—	3,946
	<u>\$ 3,946</u>	<u>\$ 26,296</u>	<u>\$ 30,242</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, 2020 are identified in the following tables:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	\$ 217,095	\$ 217,095
Asset-backed securities	9,438	9,438
Corporate debt securities	39,513	39,513
U.S. government agency bonds	53,582	53,582
Money market funds	4,427	4,427
	<u>\$ 324,055</u>	<u>\$ 324,055</u>

(in thousands)	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	\$ —	\$ 25,825	\$ 25,825
Deferred compensation plan liability	4,078	—	4,078
	<u>\$ 4,078</u>	<u>\$ 25,825</u>	<u>\$ 29,903</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, 2021 was \$4.5 million.

The Company's Senior Secured Term Loan due 2026 falls 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 due 2026 approximates the fair value.

The Company did not have any Level 3 assets as of March 31, 2021 or December 31, 2020.

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, 2021 (in thousands)	Valuation Technique	Unobservable Input	Range
Clinical and regulatory milestones	\$ 25,559	Probability weighted discounted cash flow	Discount rate	7.5%
			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, 2021 and March 31, 2020, respectively:

(in thousands)	Three Months Ended March 31,	
	2021	2020
Balance, beginning of the period	\$ 25,825	\$ 22,681
Changes in fair value during the period, included in the Consolidated Statements of Operations	471	931
Balance, end of the period	\$ 26,296	\$ 23,612

Note 9. 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,	
	2021	2020
Numerator:		
Net loss attributable to common stockholders	\$ (65,664)	\$ (88,948)
Denominator:		
Weighted average common shares outstanding — basic and diluted	264,369,317	256,968,248

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,	
	2021	2020
Options to purchase common stock	15,084	19,086
Convertible notes	458	462
Outstanding warrants, convertible to common stock	—	2,554
Unvested restricted stock units	7,466	7,562
Total number of potentially issuable shares	23,008	29,664

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 by the first, oral monotherapy for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease, 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, or cipaglucosidase alfa/miglustat), 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 initiated the rolling Biologic License Application ("BLA") submission to the FDA in the fourth quarter of 2020.

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 a research 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"), Mucopolysaccharidosis Type IIIB ("MPS IIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPS IIIA"). Our research 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. In September 2020 and February 2021, the European Medicines Agency ("EMA") granted Priority Medicines ("PRIME") designation and the FDA granted Fast Track designation, respectively, to the CLN6 Batten disease gene therapy, AT-GTX-501, for the treatment of patients with variant late infantile neuronal ceroid lipofuscinosis 6 ("vLINCL6").

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 diseases, 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 been significantly impacted from the novel coronavirus ("COVID-19") pandemic thus far. However, we continued to observe increased lag times between patient identification and Galafold[®] initiation due to the resurgence of COVID-19 into 2021. We have maintained operations in all geographies, secured our global supply chain for our commercial and clinical products, as well as maintained the operational integrity of 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, 2021, Galafold[®] revenue totaled \$66.4 million, an increase of \$5.9 million compared to the same period in the prior year. We continue to see strong commercial momentum and expansion into additional geographies. In countries where we have been operating the longest, 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.* In December 2020, we completed last patient, last visit in our global Phase 3 pivotal study of AT-GAA (ATB200-03, also known as "PROPEL") with 123 patients at 62 sites in 24 countries and initiated the rolling BLA submission to the FDA for AT-GAA. In February 2021, we subsequently reported topline results for the PROPEL study. Additionally, in 2020, orphan drug designation was received in Japan and the British Medicines and Healthcare Products Regulatory Agency issued a Promising Innovative Medicine ("PIM") designation for AT-GAA for the treatment of late-onset Pompe disease. Previously, the U.S. FDA granted BTB for AT-GAA for the treatment of late-onset Pompe disease.
- *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 a research collaboration with Penn. Some recent advances include, in February 2021, we presented initial clinical data from the Phase 1/2 CLN3 gene therapy study that suggests early signs of disease stabilization and the potential to slow the neurological disease progression in children living with CLN3 Batten disease. Additionally, in February 2021, we presented preclinical data from our Fabry disease gene therapy clinical candidate, AT-GTX-701, with an engineered GLA transgene improved for stability demonstrated greater substrate reduction than wild type constructs across all tissues and doses.
- *Manufacturing.* We continued to manufacture our Pompe biologic at commercial scale (1,000L) for our clinical studies 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 with our strategic partners to support our clinical manufacturing capabilities. Thus far, our global supply chains have not been interrupted and we have maintained our ability to manufacture Galafold[®] as well as our clinical supply during the COVID-19 pandemic.
- *Financial strength.* Total cash, cash equivalents, and marketable securities as of March 31, 2021 was \$417.4 million. Based on current operating models, we believe that the current cash position, which includes expected revenues, is sufficient to fund our operations and ongoing research programs to achieve self-sustainability. Potential impacts of the COVID-19 pandemic, 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 1,384 mutations amenable to Galafold[®] treatment, which represent up to half of all patients with Fabry disease. In countries where mutations are provided only on the amenability website, these 1,384 amenable mutations are now available. Marketing authorization approvals have 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 2021.

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 adeno-associate viral ("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. In February 2021, we presented initial preclinical data from our investigational AAV gene therapy program. This initial preclinical study assessed a range of single doses of AAV in GlA knockout mice with either wild-type hGLA ("unmodified hGLA") or an Amicus/Penn engineered hGLA transgenes ("engineered hGLA" or "AT-GTX-701"). The engineered hGLA AAV gene therapy demonstrated stable homodimer formation, enhanced temperature, plasma and neutral pH stability compared to the unmodified hGLA AAV gene therapy. In the lowest tested dose of AT-GTX-701, GlA knockout mice showed partial substrate reduction, while highest tested dose resulted in near complete substrate reduction. Additionally, AT-GTX-701 demonstrated significantly greater lyso-Gb3/GL-3 substrate reduction across all Fabry disease relevant tissues including the dorsal root ganglia ("DRG"), kidney, and heart, with reductions at low dose being equal to or greater than the reductions observed at higher doses with wildtype transgene and provided the first evidence for DRG storage reduction in a Fabry mouse model treated with an AAV gene therapy.

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, or cipaglucosidase alfa, with an optimized carbohydrate structure to enhance lysosomal uptake, administered in combination with AT2221, or miglustat, that functions as an enzyme stabilizer. The small molecule enzyme stabilizer, miglustat or AT2221, binds to and stabilizes ATB200, or cipaglucosidase alfa, preventing inactivation of rhGAA in circulation to improve the uptake of active enzyme in key disease-relevant tissues, resulting in increased clearance of accumulated substrate, glycogen. Miglustat is not an active ingredient that contributes directly to substrate reduction ("glycogen").

We initiated ATB200-03 (or "PROPEL"), a global Phase 3 clinical study of AT-GAA in adult patients with late-onset Pompe disease in December 2018 and completed last patient, last visit in December 2020. In February 2021, we reported topline results from the Phase 3 PROPEL study. Patients in PROPEL were randomized 2:1 so that for every two patients randomized to be treated with AT-GAA, one was randomized to be treated with alglucosidase alfa. Of the Pompe patients enrolled, 77% were being treated with alglucosidase alfa (n=95) immediately prior to enrollment ("Switch") and 23% had never been treated with any ERT (n=28) ("Naïve"). 117 patients completed the PROPEL study and all 117 voluntarily enrolled in the long-term extension study. The primary endpoint of the study was the mean change in 6-minute walk distance as compared with baseline measurements at 52 weeks across the combined ERT Switch and ERT Naïve patient populations. In this combined population patients taking AT-GAA (n=85) walked on average 21 meters farther at 52 weeks compared to 7 meters with those treated with alglucosidase alfa (n=37). This primary endpoint in the combined population was assessed for superiority and while numerically greater, statistical significance for superiority on this combined population was not achieved for the AT-GAA arm as compared to the alglucosidase alfa arm (p=0.072).

Per the hierarchy of the statistical analysis plan, the first key secondary endpoint of the study was the mean change in percent-predicted Forced Vital Capacity ("FVC") at 52 weeks across the combined population. In this combined population patients taking AT-GAA demonstrated a nominally statistically significant and clinically meaningful difference for superiority over those treated with alglucosidase alfa. AT-GAA significantly slowed the rate of respiratory decline in patients after 52 weeks. Patients treated with AT-GAA showed a 0.9% absolute decline in percent-predicted FVC, compared to a 4.0% absolute decline in the alglucosidase alfa arm (p=0.023). Patients within the combined study population demonstrated statistically significant improvements on the GSGC ("Gait, Stairs, Gower's Chair") key secondary endpoint, which captures strength, coordination and mobility, compared to a worsening for alglucosidase alfa treated patients in the overall population (p<0.05). Additionally, lower MMT (Manual Muscle Testing), Patient-Reported Outcomes Measurement Information System ("PROMIS") physical function and PROMIS fatigue secondary endpoints favored AT-GAA treated patients over alglucosidase alfa treated patients. Results also showed improvements in the two important biomarker endpoints of Pompe disease (Hex-4 and CK), which significantly favored AT-GAA compared to alglucosidase alfa (p<0.001). AT-GAA demonstrated a similar safety profile to alglucosidase alfa.

The PROPEL Switch patients entered the study having been treated with alglucosidase alfa for a minimum of two years. More than two thirds (67%+) of those patients had been on ERT treatment for more than five years prior to entering the PROPEL study (mean of 7.4 years). A pre-specified analysis of the patients switching from alglucosidase alfa on 6-minute walk

distance showed that after 52 weeks from switching, AT-GAA treated patients (n=65) walked 16.9 meters farther than their baseline, compared to 0.0 meters for those patients who were randomized to remain on alglucosidase alfa (n=30) (p=0.046). A pre-specified analysis of the patients switching from alglucosidase alfa on percent-predicted FVC showed that AT-GAA treated patients stabilized and slightly improved their respiratory function on this important measure while those patients remaining on alglucosidase alfa continued to significantly decline in respiratory muscle function. AT-GAA patients showed a 0.1% absolute increase in percent-predicted FVC while the alglucosidase alfa patients showed a 4.0% absolute decline over the course of the year (p=0.006).

The PROPEL Naïve patients treated with AT-GAA for 52 weeks (n=20) walked 33 meters farther than their baseline, on the 6-minute walk distance endpoint. The Naïve patients treated with alglucosidase alfa (n=7) walked 38 meters farther than their baseline. The difference between the two groups was not statistically significant (p=0.60). Additionally, patients never previously treated with any ERT showed similar declines in percent-predicted FVC at 52 weeks of -4.1% for AT-GAA treated patients and -3.6% for alglucosidase alfa treated patients. The difference between the two groups was not statistically significant (p=0.57).

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 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 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 provided initial validation for combining Amicus-engineered transgenes with Penn's AAV gene therapy technologies.

In May 2020, we presented preclinical data with the engineered hGAA AAV in single and combined central nervous system ("CNS") and systemic directed gene therapy in a mouse model of Pompe disease with advanced disease at treatment. The engineered hGAA AAV showed better targeting and clearance of glycogen storage at low doses in Pompe mice compared to unmodified hGAA AAV. High dose IV therapy showed strength rescue and the addition of high dose intracerebroventricular ("ICV") therapy to high dose IV provided incremental benefit.

Gene Therapy for Various Types of Batten Disease

Through our license with Nationwide Children's and research collaboration with Penn, 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 enrolled thirteen patients who received a single one-time intrathecal administration of AAV-CLN6 gene therapy. In October 2020, we reported additional positive interim results from our CLN6 Batten disease AAV-CLN6 gene therapy program, AT-GTX-501. Interim safety data are available for 13 patients with CLN6 Batten disease. Interim safety data demonstrated the treatment with AT-GTX-501 was generally well tolerated. The majority of adverse events ("AEs") were mild and unrelated to treatment. No pattern of adverse events related to AAV or CLN6 immunogenicity was observed. Interim efficacy data are available within the Hamburg Motor and Language scores and showed a meaningful effect in slowing disease progression for twelve patients reaching the 12-month timepoint and for eight patients up to 24 months, post-administration of the AAV-CLN6 gene therapy. Additionally, in October 2019, we reported interim clinical data that suggested stabilization of various components of Hamburg 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 an AAV serotype AT-GTX-502 gene therapy in patients 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. One patient is currently dosed in the high-dose cohort. In February 2021, we announced initial safety for the first four patients up to 15 months post-administration of AT-GTX-502 and preliminary efficacy data for the first three patients in the low-dose cohort for up to 15 months post-administration of AT-GTX-502, as well as one patient in the high-dose cohort for up to 3 months post-administration of AT-GTX-502. Initial results of the study suggest that AT-GTX-502 was well tolerated and demonstrated potential early signs of disease stabilization compared to a natural history dataset.

CDKL5 Deficiency Disorder

We are researching a potential first-in-class genetic medicine for CDD consisting of a CDKL5 protein engineered for cross correction, delivered as either a protein replacement or as a gene therapy 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 MPS IIIB as well as a next generation program in MPS IIIA. 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 to 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 and orphan diseases. We are exploring potential collaborations, alliances, and other business development opportunities on a regular basis. These opportunities may include business combinations, partnerships, the strategic out-licensing of certain assets, or the acquisitions of preclinical-stage, clinical-stage, or marketed products or platform technologies 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, 2021 compared to March 31, 2020

The following table provides selected financial information for the Company:

(in thousands)	Three Months Ended March 31,		
	2021	2020	Change
Net product sales	\$ 66,402	\$ 60,525	\$ 5,877
Cost of goods sold	6,539	6,552	(13)
Cost of goods sold as a percentage of net product sales	9.8 %	10.8 %	(1.0)%
Operating expenses:			
Research and development	64,117	89,120	(25,003)
Selling, general, and administrative	46,726	40,215	6,511
Changes in fair value of contingent consideration payable	471	931	(460)
Depreciation and amortization	1,604	1,764	(160)
Other (expense) income:			
Interest income	165	1,515	(1,350)
Interest expense	(7,992)	(3,729)	(4,263)
Other expense	(3,200)	(8,316)	5,116
Income tax expense	(1,582)	(361)	(1,221)
Net loss attributable to common stockholders	\$ (65,664)	\$ (88,948)	\$ 23,284

Net Product Sales. Net product sales increased \$5.9 million during the three months ended March 31, 2021 compared to the same period in the prior year. The increase was primarily due to continued growth in the U.S., Europe and Japan markets.

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,	
	2021	2020
Projects		
Third party direct project expenses		
Galafold® (Fabry Disease)	\$ 2,227	\$ 1,761
AT-GAA (Pompe Disease)	21,114	34,907
Gene therapy programs	13,816	21,487
Pre-clinical and other programs	68	713
Total third-party direct project expenses	37,225	58,868
Other project costs		
Personnel costs	20,179	22,718
Other costs	6,713	7,534
Total other project costs	26,892	30,252
Total research and development costs	\$ 64,117	\$ 89,120

The \$25.0 million decrease in research and development costs was primarily due to the timing of clinical research and manufacturing costs associated with the advancement of clinical studies in the Pompe program, a decrease in the gene therapy programs driven by the timing of spend of the pipeline initiatives and a decrease in personnel costs primarily due to realignment with strategic priorities.

Selling, General, and Administrative Expense. Selling, general, and administrative expense increased \$6.5 million, mainly driven by increased personnel costs.

Interest Expense. Interest expense increased \$4.3 million during the three months ended March 31, 2021 compared to the same period in the prior year. The increase was driven by the \$400 million Senior Secured Loan due 2026 entered in July 2020.

Other Expense. The \$5.1 million variance was primarily driven by foreign exchange gains in the remeasurement of our intercompany transactions.

Income Tax Expense. The income tax expense for the three months ended March 31, 2021 was \$1.6 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 jurisdictions 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, 2021, we had cash, cash equivalents, and marketable securities of \$417.4 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, 2021 was \$72.4 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2021 of \$65.7 million and an overall decrease in cash from changes from operating assets and liabilities of \$32.5 million, mainly related to the timing of contract manufacturing and research costs. This was partially offset by \$20.4 million of stock compensation and \$5.5 million of other non-cash adjustments.

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 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 Provided by Investing Activities

Net cash provided by investing activities for the three months ended March 31, 2021 was \$86.6 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 \$163.7 million for the sale and redemption of marketable securities, partially offset by \$76.2 million for the purchase of marketable securities and \$0.9 million for capital expenditures.

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 capital expenditures.

Net Cash Provided by (Used in) Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2021 was \$8.8 million. Net cash provided by financing activities primarily reflects \$19.2 million from the exercise of the remaining outstanding warrants and \$4.2 million from the exercise of stock options, partially offset by \$14.2 million from the purchase of vested restricted stock units.

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.

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 scope, progress, results and costs of our 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" or "cipaglucosidase alfa") and gene therapies;
- the future results of on-going preclinical research and subsequent clinical trials for CDD, Pompe gene therapy, Fabry gene therapy, MPS IIIB, next generation MPS IIIA, and other pipeline candidates we may identify from time to time, including our ability to obtain regulatory approvals and commercialize these therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates, including AT-GAA;
- 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") and, if our regulatory filings are accepted and approved, AT-GAA;
- 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[®] and, if our regulatory filings are accepted and approved, AT-GAA;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold[®];
- our ability to obtain market acceptance of Galafold[®] and, if our regulatory filings are accepted and approved, AT-GAA;
- 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 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. Based on current operating models, we believe that the current cash position, which includes expected revenues, is sufficient to fund our operations and ongoing research programs to achieve self-sustainability. Potential impacts of the COVID-19 pandemic, 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 - In connection with our acquisition of Celenex in 2018, we agreed to pay up to an additional \$10 million in connection with the achievement of certain development milestones, \$220 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 research 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 July 2012, as amended in November 2013, we entered into an agreement with GlaxoSmithKline ("GSK"), pursuant to which Amicus obtained global rights to develop and commercialize Galafold[®] as a monotherapy and in combination with ERT for Fabry disease ("Collaboration Agreement"). Under the terms of the Collaboration Agreement, 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.

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, 2021 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, 2020.

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 \$0.8 million as of March 31, 2021. 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, 2021, we had a \$400 million Senior Secured Term Loan due 2026 that bears interest at a rate equal to the 3-month LIBOR, subject to a 1% floor, plus 6.5% 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, 2021 was 7.5%. A hypothetical 100 basis point increase or decrease in the average interest rate on our variable rate debt would result in a \$1.0 million change in the interest expense as of March 31, 2021.

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 due 2026 in order to replace LIBOR with an alternative standard. 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. 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, 2020. There have been no material changes in our financial instrument portfolio or market risk exposures since our fiscal year ended December 31, 2020.

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 material legal proceedings.

ITEM 1A. RISK FACTORS

There have been no material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020.

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

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

The following table provides certain information with respect to purchase of our common stock during the three months ended March 31, 2021:

Period	Total Number of Shares Purchased ⁽¹⁾	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Maximum Number (or Approximate Dollar Value) of Shares That May Yet Be Purchased Under the Plans or Programs
January 1, 2021 through January 31, 2021	617,241	\$ 22.87	—	—
February 1, 2021 through February 28, 2021	7,360	\$ 12.93	—	—
March 1, 2021 through March 31, 2021	7,813	\$ 10.52	—	—
Total	632,414	\$ 22.60	—	—

⁽¹⁾ Represents shares of common stock withheld to satisfy taxes associated with the vesting of restricted stock awards

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

None

ITEM 6. EXHIBITS

Exhibit Number	Description
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</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)

**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 10, 2021

/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 10, 2021

/s/ Daphne Quimi
Daphne Quimi
Chief Financial Officer

