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, 2024

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	**71-0869350**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification Number)
47 Hulfish Street, Princeton, NJ	**08542**
(Address of Principal Executive Offices)	(Zip Code)

(609) 662-2000

(Registrant's Telephone Number, Including Area Code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, 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	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

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

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

The number of shares outstanding of the registrant's common stock, $0.01 par value per share, as of April 25, 2024 was296,198,963 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended March 31, 2024

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," "might," "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 clinical trials for our drug candidates;
- the cost of manufacturing drug supply for our commercial, clinical and preclinical studies, including the cost of manufacturing Pombiliti ® (also referred to as "ATB200" or "cipaglucosidase alfa");
- the future results of preclinical research and subsequent clinical trials for pipeline candidates we may identify from time to time, including our ability to obtain regulatory approvals and commercialize such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- any changes in regulatory standards relating to the review of our product candidates;
- any changes in laws, rules or regulations affecting our ability to manufacture, transport, test, develop, or commercialize our products, including Galafold®, Pombiliti® + Opfolda®, or our product candidates;
- the costs of commercialization activities, including product marketing, sales, and distribution;
- the emergence of competing technologies and other adverse market developments;
- the estimates regarding the potential market opportunity for our products and product candidates;
- our ability to successfully commercialize Galafold® (also referred to as "migalastat HCl");
- our ability to successfully commercialize Pombiliti® + Opfolda® (together, also referred to as "AT-GAA") in the E.U., U.K., and U.S., and elsewhere, if regulatory applications are approved;
- our ability to manufacture or supply sufficient clinical or commercial products, including Galafold ® and Pombiliti® + Opfolda®;
- our ability to obtain reimbursement for Galafold® and Pombiliti® + Opfolda®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold ® and Pombiliti® + Opfolda®;
- our ability to obtain market acceptance of Galafold® and Pombiliti® + Opfolda®, or any other product developed or acquired that has received regulatory approval;
- the costs of preparing, filing, and prosecuting patent applications and maintaining, enforcing, and defending intellectual property-related claims, including Hatch-Waxman litigation;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing or may pursue against others, including Hatch-Waxman litigation;
- the extent to which we acquire or invest in businesses, products, and technologies;
- our ability to successfully integrate acquired products and technologies into our business, or successfully divest or license existing products and technologies from 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 licensing agreements, collaborations, partnerships or other similar arrangements and to obtain milestone, royalty, or other economic benefits from any such collaborators;

- the costs associated with, and our ability to comply with, emerging environmental, social and governance standards, including climate reporting requirements at the local, state and national levels;
- our ability to successfully protect our information technology systems and maintain our global operations and supply chain without interruption;
- our ability to accurately forecast revenue, operating expenditures, or other metrics impacting profitability;
- 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, 2023, 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 herein 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 collaborations, alliances, business combinations, partnerships, strategic out-licensing of certain assets, the acquisition of preclinical-stage, clinical-stage, marketed products or platform technologies or other 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, 2023 (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, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 209,761	$ 246,994
Investments in marketable securities	29,842	39,206
Accounts receivable	76,433	87,632
Inventories	60,759	59,696
Prepaid expenses and other current assets	54,444	49,533
Total current assets	431,239	483,061
Operating lease right-of-use assets, net	23,003	26,312
Property and equipment, less accumulated depreciation of $26,563 and $25,429 at March 31, 2024 and December 31, 2023, respectively	32,421	31,667
Intangible assets, less accumulated amortization of $3,328 and $2,510 at March 31, 2024 and December 31, 2023, respectively	19,672	20,490
Goodwill	197,797	197,797
Other non-current assets	17,657	18,553
Total Assets	$ 721,789	$ 777,880
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,210	$ 15,120
Accrued expenses and other current liabilities	124,622	144,245
Operating lease liabilities	8,270	8,324
Total current liabilities	142,102	167,689
Long-term debt	388,391	387,858
Operating lease liabilities	47,831	48,877
Other non-current liabilities	12,771	13,282
Total liabilities	591,095	617,706
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 296,159,417 and 293,594,209 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	2,922	2,918
Additional paid-in capital	2,853,550	2,836,018
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	6,847	5,429
Unrealized loss on available-for-sale securities	(203)	(188)
Warrants	71	71
Accumulated deficit	(2,732,493)	(2,684,074)
Total stockholders' equity	130,694	160,174
Total Liabilities and Stockholders' Equity	$ 721,789	$ 777,880

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,	
	2024	**2023**
Net product sales	$ 110,403	$ 86,270
Cost of goods sold	13,567	6,942
Gross profit	96,836	79,328
Operating expenses:		
Research and development	28,329	41,499
Selling, general, and administrative	88,029	73,957
Changes in fair value of contingent consideration payable	—	251
Restructuring charges	6,045	—
Depreciation and amortization	2,154	1,257
Total operating expenses	124,557	116,964
Loss from operations	(27,721)	(37,636)
Other expense:		
Interest income	1,540	2,199
Interest expense	(12,436)	(11,844)
Other expense	(4,966)	(5,938)
Loss before income tax	(43,583)	(53,219)
Income tax (expense) benefit	(4,836)	287
Net loss attributable to common stockholders	$ (48,419)	$ (52,932)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.16)	$ (0.18)
Weighted-average common shares outstanding — basic and diluted	302,903,009	291,336,750

See accompanying Notes to Consolidated Financial Statements

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

	Three Months Ended March 31,	
	2024	**2023**
Net loss	$ (48,419)	$ (52,932)
Other comprehensive gain, net of tax:		
Foreign currency translation adjustment gain	1,418	5,446
Unrealized loss on available-for-sale securities	(15)	(85)
Other comprehensive gain	1,403	5,361
Comprehensive loss	$ (47,016)	$ (47,571)

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, 2024

	Common Stock		Additional Paid-In Capital	Warrants	Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31,2023	293,594,209	$ 2,918	$ 2,836,018	$ 71	$ 5,241	$ (2,684,074)	$ 160,174
Stock options exercised, net	444,497	4	3,450	—	—	—	3,454
Vesting of restricted stock units, net of taxes	2,120,711	—	(16,721)	—	—	—	(16,721)
Stock-based compensation	—	—	30,803	—	—	—	30,803
Unrealized loss on available-for-sale securities	—	—	—	—	(15)	—	(15)
Foreign currency translation adjustment	—	—	—	—	1,418	—	1,418
Net loss	—	—	—	—	—	(48,419)	(48,419)
Balance at March 31, 2024	296,159,417	$ 2,922	$ 2,853,550	$ 71	$ 6,644	$ (2,732,493)	$ 130,694

Three Months Ended March 31, 2023

	Common Stock		Additional Paid-In Capital	Warrants	Other Comprehensive Gain (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31,2022	281,108,273	$ 2,815	$ 2,664,744	$ 83	$ (12,105)	$ (2,532,490)	$ 123,047
Stock options exercised, net	384,108	3	2,652	—	—	—	2,655
Vesting of restricted stock units, net of taxes	1,612,975	—	(12,806)	—	—	—	(12,806)
Stock-based compensation	—	—	34,894	—	—	—	34,894
Issuance of shares in connection with at-the-market offering, net of issuance costs	195,229	2	2,352	—	—	—	2,354
Unrealized loss on available-for-sale securities	—	—	—	—	(85)	—	(85)
Foreign currency translation adjustment	—	—	—	—	5,446	—	5,446
Net loss	—	—	—	—	—	(52,932)	(52,932)
Balance at March 31, 2023	283,300,585	$ 2,820	$ 2,691,836	$ 83	$ (6,744)	$ (2,585,422)	$ 102,573

See accompanying Notes to Consolidated Financial Statements

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

	Three Months Ended March 31,	
	2024	**2023**
Operating activities		
Net loss	$ (48,419)	$ (52,932)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of debt discount and deferred financing	531	667
Depreciation and amortization	2,154	1,257
Stock-based compensation	30,803	34,894
Non-cash changes in the fair value of contingent consideration payable	—	251
Foreign currency remeasurement loss	1,736	5,885
Deferred taxes	—	(4,939)
Other	5,818	—
Changes in operating assets and liabilities:		
Accounts receivable	9,557	(1,367)
Inventories	(1,678)	(3,158)
Prepaid expenses and other current assets	(5,340)	1,839
Accounts payable, accrued expenses, and other current liabilities	(24,483)	(72)
Other non-current assets and liabilities	(374)	(394)
Net cash used in operating activities	$ (29,695)	$ (18,069)
Investing activities		
Sale and redemption of marketable securities	38,907	54,944
Purchases of marketable securities	(29,559)	(16,747)
Capital expenditures	(1,811)	(1,942)
Net cash provided by investing activities	$ 7,537	$ 36,255
Financing activities		
Payment of finance leases	(42)	(28)
Withholding taxes paid on vested restricted stock units	(16,721)	(12,806)
Proceeds from stock options exercised, net	3,454	2,655
Proceeds from the issuance of shares in connection with at-the-market offering, net of issuance costs	—	2,354
Net cash used in financing activities	$ (13,309)	$ (7,825)
Effect of exchange rate changes on cash, cash equivalents, and restricted cash	$ (1,816)	$ 1,507
Net (decrease) increase in cash, cash equivalents, and restricted cash at the end of the period	(37,283)	11,868
Cash, cash equivalents, and restricted cash at the beginning of period	250,077	153,115
Cash, cash equivalents, and restricted cash at the end of period	$ 212,794	$ 164,983
Supplemental disclosures of cash flow information		
Cash paid during the period for interest	$ 11,801	$ 11,361
Capital expenditures unpaid at the end of period	$ 1,110	$ 1,260
Cash paid for taxes	$ 635	$ 178
Tenant improvements paid through lease incentives	$ 52	$ —

See accompanying Notes to Consolidated Financial Statements

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

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 seeks to deliver the highest quality therapies that have the potential to obsolete current treatments, provide significant benefits to patients, and be first- or best-in-class. The Company's two marketed therapies are Galafold®, the first oral monotherapy for people living with Fabry disease who have amenable genetic variants, and Pombiliti® + Opfolda®, a novel treatment designed to improve uptake of active enzyme into key disease relevant tissues for adults living with late-onset Pompe disease.

Galafold® (also referred to as "migalastat"), is approved in over 40 countries around the world, including the United States ("U.S."), European Union ("E.U."), United Kingdom ("U.K."), and Japan. Additionally, Galafold® has been granted orphan drug designation in the U.S., E.U., U.K., Japan and several other countries.

Pombiliti® + Opfolda® (also referred to as "cipaglucosidase alfa-atga/miglustat"), was approved in 2023 in the three largest Pompe markets: the U.S., the E.U., and the U.K. Multiple regulatory submissions and reimbursement processes with global health authorities are currently underway. Additionally, Pombiliti® + Opfolda® has been granted orphan drug designation in the U.S., E.U., U.K., Japan and several other countries.

The Company had an accumulated deficit of $ 2.7 billion as of March 31, 2024 and anticipates incurring losses through the fiscal year ending December 31, 2024. The Company has historically funded its operations through stock offerings, product revenues, debt issuances, collaborations, and other financing arrangements.

Based on its current operating model, the Company believes the current cash position, which includes expected revenues, is sufficient to fund the Company's operations and ongoing research programs for at least the next 12 months. Potential business development opportunities, pipeline expansion, and investment in manufacturing capabilities could impact the Company's long-term capital requirements.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited Consolidated Financial Statements have been prepared 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, certain financial information that is normally included in annual financial statements prepared in accordance with U.S. GAAP, but that is not required for interim reporting purposes, has been omitted. In the opinion of management, the accompanying unaudited Consolidated Financial Statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's interim financial information. Management has determined that the Company operates in one segment focused on the discovery, development, and commercialization of advanced therapies to treat a range of devastating rare and orphan diseases.

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, 2023. 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, 2023.

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 weighted 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. Transactions which are not in the functional currency of the entity are remeasured into the functional currency with gains or losses resulting from the remeasurement recorded in other expense.

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.

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 other comprehensive gain in the Company's Consolidated 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 as a component of 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, marketable securities, and accounts receivable.

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's accounts receivable at March 31, 2024 have primarily arisen from Galafold ® sales in Europe, the U.S., and Japan. The Company periodically assesses 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, 2024, the Company's allowance for doubtful accounts was $0.1 million.

Revenue Recognition

The Company has recorded revenue on sales where its products are available either on a commercial basis or through a reimbursed early access program. Product orders are generally received from distributors and pharmacies, with the ultimate payor often a government authority.

The Company recognizes revenue when its performance obligation to its customers have been satisfied, which occurs at a point in time when the pharmacies or distributors obtain control of the products. 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 primarily consist of third-party discounts and rebates. The identified variable consideration is recorded as a reduction of revenue at the time revenue from the sale 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 disaggregated by product:

(in thousands)	Three Months Ended March 31, 2024	2023
Galafold®	$ 99,359	$ 86,112
Pombiliti® + Opfolda®	11,044	158
Total net product sales	$ 110,403	$ 86,270

The following table summarizes the Company's net product sales disaggregated by geographic area:

(in thousands)	Three Months Ended March 31, 2024	2023
U.S.	$ 37,375	$ 28,831
Ex-U.S.	73,028	57,439
Total net product sales	$ 110,403	$ 86,270

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 Company's Consolidated Statements of Operations.

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

Recent Accounting Developments - Guidance Adopted in 2024

In November 2023, the Financial Accounting Standards Board ("FASB") issued the Accounting Standards Update ("ASU") No. 2023-07, Segment Reporting (Topic 280): *Improvements to Reportable Segment Disclosures*. The amendments expand reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. The amendments require, among other things, disclosure of the title and position of the chief operating decision maker and require that public entities with a single reportable segment provide all disclosures required by this update and existing segment disclosures in Topic 280. Annual disclosures are required for fiscal years beginning after December 15, 2023 and interim disclosures are required for periods within fiscal years beginning after December 15, 2024. Retrospective application is required unless it is impracticable, and early adoption is permitted. The Company adopted this guidance on January 1, 2024. This ASU applies to disclosure requirements only, and the Company will provide required annual disclosures as part of the 2024 Annual Report on Form 10-K and required interim disclosures as part of 2025 Quarterly Reports on Form 10-Q.

Recent Accounting Developments - Guidance Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, Income Taxes (Topic 740): I *mprovements to Income Tax Disclosures.* The ASU requires disclosure of disaggregated income taxes paid, prescribes standard categories for the components of the effective tax rate reconciliation, and modifies other income tax-related disclosures. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024, must be applied prospectively with an option to apply retrospectively, and early adoption is permitted. The adoption of this guidance is not expected to have a significant impact on the Company's consolidated financial statements.

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

As of March 31, 2024, the Company held $ 209.8 million in cash and cash equivalents and $ 29.8 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 other comprehensive gain in the Consolidated 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 the Consolidated Statements of Operations 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 the Consolidated Statements of Operations.

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, in accordance with Company policy, securities are of high 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:

	As of March 31, 2024			
(in thousands)	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	$ 209,761	$ —	$ —	$ 209,761
Commercial paper	25,718	—	(4)	25,714
U.S. government agency bonds	3,978	—	(1)	3,977
Money market	100	—	—	100
Certificates of deposit	51	—	—	51
	$ 239,608	$ —	$ (5)	$ 239,603
Included in cash and cash equivalents	$ 209,761	$ —	$ —	$ 209,761
Included in marketable securities	29,847	—	(5)	29,842
Total cash, cash equivalents, and marketable securities	$ 239,608	$ —	$ (5)	$ 239,603

	As of December 31, 2023			
(in thousands)	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	$ 246,994	$ —	$ —	$ 246,994
Commercial paper	14,651	12	—	14,663
Treasury bill	12,944	2	—	12,946
U.S. government agency bonds	11,450	—	(4)	11,446
Money market	100	—	—	100
Certificate of deposit	51	—	—	51
	$ 286,190	$ 14	$ (4)	$ 286,200
Included in cash and cash equivalents	$ 246,994	$ —	$ —	$ 246,994
Included in marketable securities	39,196	14	(4)	39,206
Total cash, cash equivalents, and marketable securities	$ 286,190	$ 14	$ (4)	$ 286,200

For both the three months ended March 31, 2024 and March 31, 2023, there were no realized gains or losses. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the marketable securities as of March 31, 2024 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 as a component of other comprehensive gain. The fair value of these marketable securities in unrealized loss positions are $29.7 million and $11.4 million as of March 31, 2024 and December 31, 2023, 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.

	As of March 31,	
(in thousands)	**2024**	**2023**
Cash and cash equivalents	$ 209,761	$ 160,602
Restricted cash	3,033	4,381
Cash, cash equivalents, and restricted cash shown in the Consolidated Statements of Cash Flows	$ 212,794	$ 164,983

4. Inventories

The following table summarizes the components of the Company's inventories for each of the periods indicated:

(in thousands)	**March 31, 2024**	**December 31, 2023**
Raw materials	$ 31,613	$ 30,230
Work-in-process	22,146	22,597
Finished goods	7,000	6,869
Total inventories	$ 60,759	$ 59,696

The Company's reserve for inventory was $2.9 million and $0.5 million as of March 31, 2024 and December 31, 2023, respectively.

5. Debt

The following table summarizes the Company's debt for each of the periods indicated:

(in thousands)	**March 31, 2024**	**December 31, 2023**
Senior Secured Term Loan due 2029:		
Principal	$ 400,000	$ 400,00
Less: debt discount [(1)]	(9,230)	(9,65
Less: deferred financing [(1)]	(2,379)	(2,49
Net carrying value of Long-term debt	$ 388,391	$ 387,85

[(1)] Included in the Company's Consolidated Balance Sheets within long-term debt and amortized to interest expense over the remaining life of the Senior Secured Term Loan due 2029 using the effective interest rate method.

Interest Expense

The following table sets forth interest expense recognized related to the Company's debt for the three months ended March 31, 2024 and 2023, respectively:

	Three Months Ended March 31,	
(in thousands)	**2024**	**2023**
Contractual interest expense	$ 11,974	$ 11,230
Amortization of debt discount	$ 422	$ 381
Amortization of deferred financing	$ 109	$ 286

6. Restructuring

In the first quarter of 2024, restructuring charges are primarily related to an initiative to reduce operating costs by abandoning one of the Company's leases that it no longer believes is necessary to conduct its operations.

The associated liabilities are recorded in accrued expenses and other current liabilities in the Company's Consolidated Balance Sheets. Total charges incurred are summarized as follows:

(in thousands)	**Other Facility and Non-Lease Costs**	**Accelerated Depreciation**	**Total**
Balance as of December 31, 2023	**$ —**	**$ —**	**$ —**
Restructuring charges	3,201	2,844	6,045
Non-cash items	—	(2,844)	(2,844)
Cash settled	(310)	—	(310)
Balance as of March 31, 2024	$ 2,891	$ —	$ 2,891

7. Stock-Based Compensation

The 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 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 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 were estimated on the date of grant using a Black-Scholes option pricing model with the following weighted-average assumptions:

	Three Months Ended March 31,	
	2024	**2023**
Expected stock price volatility	57.2 %	59.3 %
Risk free interest rate	4.0 %	3.9 %
Expected life of options (years)	5.6	5.5
Expected annual dividend per share	$ —	$ —

A summary of the Company's stock options for the three months ended March 31, 2024 were as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Years	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Options outstanding, December 31, 2023	23,002	$ 11.69		
Granted	4,033	$ 14.23		
Exercised	(439)	$ 7.86		
Forfeited	(271)	$ 12.56		
Expired	(20)	$ 17.28		
Options outstanding, March 31, 2024	26,305	$ 12.13	6.4	$ 23
Vested and unvested expected to vest, March 31, 2024	24,409	$ 12.08	6.2	$ 23
Exercisable at March 31, 2024	15,979	$ 11.60	4.9	$ 22

As of March 31, 2024, the total unrecognized compensation cost related to non-vested stock options granted was $ 52.5 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, 2024 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Non-vested units as of December 31, 2023	10,033	$ 13.37		
Granted	3,251	$ 15.35		
Vested	(3,332)	$ 13.49		
Forfeited	(393)	$ 14.66		
Non-vested units as of March 31, 2024	9,559	$ 13.92	2.5	$ 112

As of March 31, 2024, there was $ 77.7 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 Company's Consolidated Statements of Operations related to the equity awards:

	Three Months Ended March 31,	
(in thousands)	2024	2023
Research and development expense	$ 4,871	$ 8,490
Selling, general, and administrative expense	25,932	26,404
Total equity compensation expense	$ 30,803	$ 34,894

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, 2024 are identified in the following tables:

(in thousands)	Level 1	Level 2	Total
Assets:			
Commercial paper	$ —	$ 25,714	$ 25,714
U.S. government agency bonds	—	3,977	3,977
Money market	7,201	—	7,201
	$ 7,201	$ 29,691	$ 36,892

(in thousands)	Level 1	Level 2	Total
Liabilities:			
Deferred compensation plan liability	$ 7,101	$ —	$ 7,101
	$ 7,101	$ —	$ 7,101

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, 2023 are identified in the following tables:

(in thousands)	Level 1	Level 2	Total
Assets:			
Commercial paper	$ —	$ 14,663	$ 14,663
Treasury bill	—	12,946	12,946
U.S. government agency bonds	—	11,446	11,446
Money market	7,631	—	7,631
	$ 7,631	$ 39,055	$ 46,686

(in thousands)	Level 1	Level 2	Total
Liabilities:			
Deferred compensation plan liability	7,531	—	7,531
	$ 7,531	$ —	$ 7,531

Deferred compensation plan liability is recorded as a component of other non-current liabilities on the Company's Consolidated Balance Sheets. The Company did not have any Level 3 assets or liabilities as of March 31, 2024 or December 31, 2023.

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.

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:

	Three Months Ended March 31,	
(in thousands, except per share amounts)	**2024**	**2023**
Numerator:		
Net loss attributable to common stockholders	$ (48,419)	$ (52,932)
Denominator:		
Weighted average common shares outstanding — basic and diluted	302,903,009	291,336,750

Dilutive common stock equivalents would include the dilutive effect of outstanding common stock options and unvested RSUs. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect. Weighted average common shares outstanding includes outstanding pre-funded warrants with an exercise price of $0.01.

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

	As of March 31,	
(in thousands)	**2024**	**2023**
Options to purchase common stock	26,305	23,43
Unvested restricted stock units	9,559	9,43
Total number of potentially issuable shares	35,864	32,86

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

The following discussion should be read in conjunction with the unaudited Consolidated Financial Statements and the notes thereto included in this Quarterly Report on Form 10-Q and the audited Consolidated Financial Statements and the notes thereto included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023. Some of the statements we make in this section are forward-looking statements within the meaning of the federal securities laws. For a complete discussion of forward-looking statements, see the section in this Quarterly Report on Form 10-Q entitled "Special Note Regarding Forward-Looking Statements". Certain risk factors may cause actual results, performance or achievements to differ materially from those expressed or implied by the following discussion. For a discussion of such risk factors, see the section in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023 entitled "Risk Factors".

Overview

We are a global, patient-dedicated biotechnology company focused on discovering, developing, and delivering novel medicines for rare diseases. We seek to deliver the highest quality therapies that have the potential to obsolete current treatments, provide significant benefits to patients, and be first- or best-in-class. Our two marketed therapies are Galafold®, the first oral monotherapy for people living with Fabry disease who have amenable genetic variants, and Pombiliti® + Opfolda®, a novel treatment designed to improve uptake of active enzyme into key disease relevant tissues for adults living with late-onset Pompe disease.

Galafold® (also referred to as "migalastat") is approved in over 40 countries around the world, including the United States ("U.S."), European Union ("E.U."), United Kingdom ("U.K."), and Japan. Additionally, Galafold® has been granted orphan drug designation in the U.S., E.U., U.K., Japan and several other countries.

Pombiliti® + Opfolda® (also referred to as "cipaglucosidase alfa-atga/miglustat") was approved in 2023 in the three largest Pompe markets: the U.S., the E.U., and the U.K. Multiple regulatory submissions and reimbursement processes with global health authorities are currently underway. Additionally, Pombiliti® + Opfolda® has been granted orphan drug designation in the U.S., E.U., U.K., Japan and several other countries.

Our Strategy

Our strategy is to create, manufacture, test, and deliver the highest quality medicines for people living with rare diseases through internally developed, jointly developed, acquired, or in-licensed products and product candidates. We are leveraging our global capabilities to develop and broaden our franchises in Fabry and Pompe disease, with focused discovery work on next generation therapies and novel technologies.

Highlights of our progress include:

- *Commercial and regulatory success in Fabry disease.* For the three months ended March 31, 2024, Galafold® revenue was $99.4 million of consolidated revenue, which represented an increase of $13.2 million compared the same period in the prior year. We continue to see strong commercial momentum and expansion into additional geographies.
- *Pompe disease program milestones.* For the three months ended March 31, 2024, Pombiliti® + Opfolda® revenue was $11.0 million of consolidated revenue. Pombiliti® + Opfolda® were approved by the European Commission ("EC") in June 2023, the Medicines and Healthcare products Regulatory Agency ("MHRA)" of the United Kingdom in August 2023, and the U.S. Food and Drug Administration ("FDA") in September 2023.
- *Pipeline advancement and growth.* We are leveraging our global capabilities to develop and broaden our franchises in Fabry and Pompe disease, with focused discovery work on next generation therapies and novel technologies.
- *Financial strength*. Total cash, cash equivalents, and marketable securities as of March 31, 2024 was $239.6 million.

Our Commercial Products and Product Candidates

Galafold® (migalastat HCl) for Fabry Disease

Our oral precision medicine Galafold® was granted accelerated approval by the FDA in August 2018 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. 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 variant. Marketing authorization approvals as well as approvals for adolescents aged 12 years and older weighing 45 kg or more have been granted in over 40 countries around the world. We plan to continue to launch Galafold® in additional countries, including for adolescents aged 12 years and older.

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 Good Laboratory Practice ("GLP") cell-based amenability assay.

Next Generation for Fabry Disease

We are committed to continued innovation for all people living with Fabry disease. As part of our long-term commitment, we are also continuing discovery for next-generation genetic medicines for Fabry disease.

Pombiliti® (cipaglucosidase alfa-atga) + Opfolda® (miglustat) for Pompe Disease

We have leveraged our biologics capabilities to develop Pombiliti® + Opfolda®, a novel treatment paradigm for late-onset Pompe disease. Pombiliti® + Opfolda® were approved by the EC in June 2023, the MHRA in August 2023, and the FDA in September 2023. Additional regulatory submissions and reimbursement processes with global health authorities are currently underway.

Pombiliti® + Opfolda® consists of a uniquely engineered rhGAA enzyme, cipaglucosidase alfa-atga, with an optimized carbohydrate structure to enhance lysosomal uptake, administered in combination with miglustat that functions as an enzyme stabilizer. Miglustat binds to and stabilizes cipaglucosidase alfa-atga reducing inactivation of rhGAA in circulation to improve the uptake of active enzyme into key disease relevant tissues. Miglustat is not an active ingredient that contributes directly to glycogen reduction.

In addition, clinical studies are ongoing in pediatric patients for both the late-onset Pompe disease ("LOPD") and infantile-onset Pompe disease ("IOPD") populations.

Next Generation for Pompe Disease

We are committed to continued innovation for all people living with Pompe disease. As part of our long-term commitment, we are also continuing discovery for next-generation genetic medicines for Pompe disease.

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 and intellectual property necessary to develop and market technologies or products with a focus on rare and orphan diseases. We are exploring potential collaborations, alliances, and various other business development opportunities on a regular basis. These opportunities may include business combinations, partnerships, the strategic out-licensing of certain assets, or the acquisition of preclinical-stage, clinical-stage, or marketed products or novel 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, 2024 compared to March 31, 2023

The following table provides selected financial information for the Company:

(in thousands)	Three Months Ended March 31, 2024		2023		Change
Net product sales	$ 110,403		$ 86,270		$ 24,133
Cost of goods sold	13,567		6,942		6,625
Cost of goods sold as a percentage of net product sales	12.3	%	8.0	%	4.3
Operating expenses:					
Research and development	28,329		41,499		(13,170)
Selling, general, and administrative	88,029		73,957		14,072
Changes in fair value of contingent consideration payable	—		251		(251)
Restructuring charges	6,045		—		6,045
Depreciation and amortization	2,154		1,257		897
Other expense:					
Interest income	1,540		2,199		(659)
Interest expense	(12,436)		(11,844)		(592)
Other expense	(4,966)		(5,938)		972
Income tax (expense) benefit	(4,836)		287		(5,123)
Net loss attributable to common stockholders	$ (48,419)		$ (52,932)		$ 4,513

Net Product Sales. Net product sales increased $24.1 million during the three months ended March 31, 2024 compared to the same period in the prior year. The increase was primarily due to both the continued growth of Galafold® in the U.S. and Europe as well as the launch of Pombiliti ® + Opfolda® in Europe and the U.S.

Cost of goods sold. Cost of goods sold includes manufacturing costs as well as royalties associated with net product sales. Cost of goods sold as a percentage of net product sales increased 4.3% primarily due to inventory write-offs associated with validation efforts in the current period.

Research and Development Expense. The following table summarizes our principal development programs and the out-of-pocket, third-party expenses incurred:

(in thousands)	Three Months Ended March 31,	
Projects	**2024**	**2023**
Third party direct project expenses		
Galafold® (Fabry Disease)	$ 1,815	$ 2,644
Pombiliti® + Opfolda® (Pompe Disease)	11,320	15,776
Pre-clinical and other programs	617	600
Total third-party direct project expenses	13,752	19,020
Other project costs		
Personnel costs	11,905	18,252
Other costs	2,672	4,227
Total other project costs	14,577	22,479
Total research and development costs	$ 28,329	$ 41,499

The $13.2 million decrease in research and development costs was primarily driven by our Pombiliti ® + Opfolda® commercial launch, decreasing both clinical spend and the number of employees supporting research and development efforts.

Selling, General, and Administrative Expense. Selling, general, and administrative expense increased $14.1 million, primarily driven by personnel costs resulting from an increase in the number of employees to support our commercial launch activities, post-approval costs not otherwise included in research and development expenses, and third-party professional fees.

Restructuring Charges. In the first quarter of 2024, restructuring charges were primarily related to an initiative to reduce operating costs by abandoning a lease that we no longer believe is useful in our operations.

Income Tax Expense . We are subject to income taxes in various jurisdictions. Our tax liabilities are largely dependent on the distributions 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® and Pombiliti® + Opfolda®, 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, product revenues, debt issuance, collaborations, and other financing arrangements.

Sources of Liquidity

In November 2022, we entered into a Sales Agreement with The Goldman Sachs & Co. LLC to create an at-the-market equity program ("ATM program"), pursuant to which we may offer to sell shares of our common stock having an aggregate offering gross proceeds of up to $250.0 million. As of March 31, 2024, an aggregate of $184.4 million worth of shares remain available to be issued and sold under the ATM program.

Cash Flow Discussion

As of March 31, 2024, we had cash, cash equivalents, and marketable securities of $239.6 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, 2024 was $29.7 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2024 of $48.4 million and a net decrease in changes in operating assets and liabilities of $22.3 million offset by $30.8 million of stock compensation and $10.2 million of other non-cash adjustments, . The changes in operating assets and liabilities was primarily due to a decrease in accounts payable and accrued expenses of $24.5 million associated with payments for Pombiliti® + Opfolda® launch activities and personnel costs.

Net cash used in operations for the three months ended March 31, 2023 was $18.1 million. The components of net cash used in operations included the net loss for the three months ended March 31, 2023 of $52.9 million offset by $34.9 million of stock compensation, $3.1 million of other non-cash adjustments, and a net increase in changes in operating assets and liabilities of $3.2 million primarily due to an increase in inventory.

Net Cash Provided by Investing Activities

Net cash provided by investing activities for the three months ended March 31, 2024 was $7.5 million. Our investing activities have consisted primarily of purchases, sales, and maturities of investments and capital expenditures. Net cash provided by investing activities reflects $38.9 million from the sale and redemption of marketable securities, partially offset by $29.6 million for the purchase of marketable securities and $1.8 million for capital expenditures.

Net cash provided by investing activities for the three months ended March 31, 2023 was $36.3 million. Our investing activities have consisted primarily of purchases, sales and maturities of investments and capital expenditures. Net cash provided

by investing activities reflects $54.9 million from the sale and redemption of marketable securities, partially offset by $16.7 million for the purchase of marketable securities and $1.9 million for capital expenditures.

Net Cash Used in Financing Activities

Net cash used in financing activities for the three months ended March 31, 2024 was $13.3 million. Net cash used in financing activities primarily reflects the withholding taxes paid on vested restricted stock units of $16.7 million, partially offset by proceeds from the exercise of stock options of $3.5 million.

Net cash used in financing activities for the three months ended March 31, 2023 was $7.8 million. Net cash used in financing activities primarily reflects the purchase of vested restricted stock units of $12.8 million, partially offset by $2.7 million of proceeds from the exercise of stock options and $2.4 million of proceeds from the issuance of shares in connection with the ATM program offering, net of issuance costs.

Funding Requirements

We expect to continue to incur significant costs in 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 clinical trials for our drug candidates;
- the cost of manufacturing drug supply for our commercial, clinical and preclinical studies, including the cost of manufacturing Pombiliti ® (also referred to as "ATB200" or "cipaglucosidase alfa");
- the future results of preclinical research and subsequent clinical trials for pipeline candidates we may identify from time to time, including our ability to obtain regulatory approvals and commercialize such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- any changes in regulatory standards relating to the review of our product candidates;
- any changes in laws, rules or regulations affecting our ability to manufacture, transport, test, develop, or commercialize our products, including Galafold®, Pombiliti® + Opfolda®, or our product candidates;
- the costs of commercialization activities, including product marketing, sales, and distribution;
- the emergence of competing technologies and other adverse market developments;
- the estimates regarding the potential market opportunity for our products and product candidates;
- our ability to successfully commercialize Galafold® (also referred to as "migalastat HCl");
- our ability to successfully commercialize Pombiliti® + Opfolda® (together, also referred to as "AT-GAA") in the E.U., U.K., and U.S., and elsewhere, if regulatory applications are approved;
- our ability to manufacture or supply sufficient clinical or commercial products, including Galafold ® and Pombiliti® + Opfolda®;
- our ability to obtain reimbursement for Galafold® and Pombiliti® + Opfolda®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold ® and Pombiliti® + Opfolda®;
- our ability to obtain market acceptance of Galafold® and Pombiliti® + Opfolda® or any other product developed or acquired that has received regulatory approval;
- the costs of preparing, filing, and prosecuting patent applications and maintaining, enforcing, and defending intellectual property-related claims, including Hatch-Waxman litigation;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing or may pursue against others, including Hatch-Waxman litigation;
- 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, or successfully divest or license existing products and technologies from 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 licensing agreements, collaborations, partnerships or other similar arrangements and to obtain milestone, royalty, or other economic benefits from any such collaborators;
- the costs associated with, and our ability to comply with, emerging environmental, social and governance standards, including climate reporting requirements at the local, state and national levels;
- our ability to successfully protect our information technology systems and maintain our global operations and supply chain without interruption;
- our ability to accurately forecast revenue, operating expenditures, or other metrics impacting profitability;
- fluctuations in foreign currency exchange rates; and
- changes in accounting standards.

We may seek additional funding through public or private financings of debt or equity. Based on our current operating model, we believe that the current cash position, which includes expected revenues, is sufficient to fund our operations and ongoing research programs for at least the next 12 months. Potential impacts of business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact our long-term capital requirements.

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 and make changes when necessary. 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, 2024 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, 2023.

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

Our market risks, and the way we manage them, are summarized in Part II, Item 7A, "Quantitative and Qualitative Disclosures About Market Risk" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023. As of March 31, 2024, there have been no material changes to our market risks or to our management of such risks since December 31, 2023.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure 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

In the fourth quarter of 2022, the Company received Paragraph IV Certification Notice Letters from Teva Pharmaceuticals USA, Inc. ("Teva"), Aurobindo Pharma Limited ("Aurobindo"), and Lupin Limited ("Lupin") in connection with Abbreviated New Drug Applications (“ANDA”) filed with the FDA requesting approval to market generic Galafold®. In November 2022, the Company filed four lawsuits against Teva, Lupin, and Aurobindo in the U.S. District Court for the District of Delaware for infringement of its Orange Book-listed patents. In the fourth quarter of 2023, a stipulation order to stay litigation with respect to Lupin was ordered. Additionally, in the first quarter of 2024, a stipulation was filed with the court and approved by the presiding judge, whereby the parties agreed to accept the Company’s definition of the terms that were in dispute. As such, the scheduled Markman hearing was deemed unneeded and cancelled. The Company has, and will continue to, vigorously enforce its Galafold® intellectual property rights.

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, 2023.

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, 2024:

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, 2024 through January 31, 2024	852,344	$ 13.60	—	—
February 1, 2024 through February 29, 2024	125,353	$ 13.76	—	—
March 1, 2024 through March 31, 2024	229,947	$ 12.00	—	—
Total	1,207,644	$ 13.31	—	—

[1] Represents shares of common stock withheld to satisfy taxes associated with the vesting of restricted stock units

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

Rule 10b5-1 Trading Plans

For the quarterly period covered by this report, no director or officer (as defined in Rule 16a-1(f) under the Exchange Act) has adopted, modified, or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

ITEM 6. EXHIBITS

Exhibit Number	Description
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.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)

SIGNATURES

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

AMICUS THERAPEUTICS, INC.

Date: May 9, 2024

By: /s/ Bradley L. Campbell
Bradley L. Campbell
President and Chief Executive Officer
(Principal Executive Officer)

Date: May 9, 2024

By: /s/ Simon Harford
Simon Harford
Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

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

I, Bradley L. Campbell, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2024

/s/ Bradley L. Campbell
Bradley L. Campbell
President and Chief Executive Officer

Exhibit 31.2

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

I, Simon Harford, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2024

/s/ Simon Harford
Simon Harford
Chief Financial Officer

Exhibit 32.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his or her capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his or her knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended March 31, 2024, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: May 9, 2024 By: /s/ Bradley L. Campbell
Bradley L. Campbell
President and Chief Executive Officer

Date: May 9, 2024 By: /s/ Simon Harford
Simon Harford
Chief Financial Officer