

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

FORM 8-K

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

Date of Report (Date of earliest event reported): February 28, 2024

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware  
(State or Other Jurisdiction  
of Incorporation)

001-33497  
(Commission  
File Number)

71-0869350  
(I.R.S. Employer  
Identification No.)

47 Hulfish Street, Princeton, NJ 08542  
(Address of Principal Executive Offices, and Zip Code)

609-662-2000  
Registrant's Telephone Number, Including Area Code

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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	FOLD	NASDAQ

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2).  
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.

## Item 2.02 – Results of Operations and Financial Condition

On February 28, 2024, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal year ended December 31, 2023. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on February 28, 2024 to discuss its full year results of operations. A copy of the conference call presentation materials is attached hereto as Exhibit 99.2. Both exhibits are incorporated herein by reference.

In accordance with General Instruction B.2. of Form 8-K, the information in this Current Report on Form 8-K and the Exhibits shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

## Item 9.01 Financial Statements and Exhibits

### (d) Exhibits:

<u>Exhibit No.</u>	<u>Description</u>
<a href="#">99.1</a>	<a href="#">Press Release, dated February 28, 2024</a>
<a href="#">99.2</a>	<a href="#">February 28, 2024 Conference Call Presentation Materials</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

**Signature Page**

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

AMICUS THERAPEUTICS, INC.

Date: February 28, 2024

By: /s/ Ellen S. Rosenberg  
Name: Ellen S. Rosenberg  
Title: Chief Legal Officer and Corporate Secretary

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**Amicus Therapeutics Announces Full-Year 2023 Financial Results and Corporate Updates**

*2023 Total Revenue of \$399.4M, a 21% Increase Year-over-Year*

*Strong Patient Demand Continues for Pombiliti™ + Opfolda™ in the U.S., U.K., and Germany*

*Projecting 2024 Galafold® Revenue Growth of 11-16% at CER*

*Anticipating Full-Year Non-GAAP Profitability in 2024*

*Conference Call and Webcast Today at 8:30 a.m. ET*

PRINCETON, NJ, Feb. 28, 2024 – Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced financial results for the full-year ended December 31, 2023.

“In 2023, Amicus made tremendous progress across all our strategic priorities,” said Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc. “We strengthened our leadership position in Fabry and Pompe disease globally and achieved our goal of non-GAAP profitability in the fourth quarter. Patient demand for Galafold exceeded our expectations and grew at the highest rate seen in the last four years, and we continue to be excited by the long-term growth potential of this important medicine. We also successfully launched our second commercial therapy, Pombiliti + Opfolda, in the three largest Pompe disease markets. In 2024, we will continue to drive significant top line revenue growth supported by sustained double-digit Galafold performance and the successful ongoing global commercial launch of Pombiliti + Opfolda, putting us on track for our first full year of non-GAAP profitability. Amicus is at a major inflection point and strongly positioned to continue to advance our mission of delivering groundbreaking new medicines to thousands of people living with rare diseases and creating value for our shareholders.”

**Corporate Highlights:**

• **Total revenues for the full-year 2023 were \$399.4 million**, up 21%, reflecting operational growth measured at constant exchange rates (CER)<sup>1</sup> of 20% and favorable currency impact of \$2.7 million or 1%. Fourth quarter total revenues were \$115.1 million, up 31%, or 27% at CER.

(in thousands)	Three Months Ended December 31,		Year over Year % Growth		Twelve Months Ended December 31,		Year over Year % Growth	
	2023	2022	Reported	at CER <sup>1</sup>	2023	2022	Reported	at CER <sup>1</sup>
Galafold®	106,600	87,989	21%	18%	387,777	329,046	18%	17%
Pombiliti™ + Opfolda™	8,482	107	n/a	n/a	11,579	187	n/a	n/a
Net Product Revenues	\$ 115,082	\$ 88,096	31%	27%	\$ 399,356	\$ 329,233	21%	20%

• **Galafold (migalastat) net product sales for the full-year 2023 were \$387.8 million**, representing a year-over-year increase of 18%, or 17% at CER. Fourth quarter net product sales were \$106.6 million. At the end of 2023, there were >2,400 people living with Fabry disease on Galafold following a year of increased demand.

• **Pombiliti (cipaglucosidase alfa-atga) + Opfolda (miglustat) net product sales for the full-year 2023 were \$11.6 million**. Fourth quarter net product sales were \$8.5 million. The commercial launch of Pombiliti + Opfolda is underway in the three largest markets with 120 patients on treatment with commercial product or scheduled to be treated as of early January and continued strong patient demand.

• **Eleven posters and an oral presentation highlighting Amicus' development programs in Fabry disease and Pompe disease presented at the 20th Annual WORLDSymposium™**. Pombiliti (cipaglucosidase alfa-atga) + Opfolda (miglustat) honored with the 2024 New Treatment Award, which recognizes important achievements in advancing new treatments approved for lysosomal diseases.

• **On a GAAP basis, net loss in the fourth quarter of 2023 was \$33.8 million**. The Company achieved non-GAAP profitability<sup>3</sup> in the fourth quarter of 2023 of \$2.6 million.



## **Full-Year 2023 Financial Results**

- Total revenue in the full-year 2023 was \$399.4 million, a year-over-year increase of 21% from total revenue of \$329.2 million in the full-year 2022. On a constant currency basis, full-year 2023 total revenue growth was 20%. Reported revenue had a favorable currency impact of approximately \$2.7 million, or 1%.
- Total GAAP operating expenses of \$439.2 million for the full-year 2023 decreased by 13% as compared to \$502.8 million for the full-year 2022.
- Total non-GAAP operating expenses of \$341.6 million for the full-year 2023 decreased by 17% as compared to \$413.2 million for the full-year 2022.
- GAAP net loss was \$151.6 million, or \$0.51 per share, for the full-year 2023, and was reduced compared to a net loss of \$236.6 million, or \$0.82 per share, for the full-year 2022.
- Non-GAAP net loss was \$38.5 million, or \$0.13 per share, for the full-year 2023, and was reduced compared to a net loss of \$152.5 million, or \$0.53 per share, for the full-year 2022.
- Cash, cash equivalents, and marketable securities totaled \$286.2 million at December 31, 2023, compared to \$293.6 million at December 31, 2022.

## **2024 Financial Guidance**

- For the full-year 2024, the Company anticipates total Galafold revenue growth between 11% and 16% at CER<sup>1</sup> driven by continued underlying demand from both switch and treatment-naïve patients, geographic expansion, label extensions, the continued diagnosis of new Fabry patients, and commercial execution across all major markets, including the U.S., EU, U.K., and Japan.
- Non-GAAP operating expense guidance for the full-year 2024 is \$345 million to \$365 million, driven by disciplined expense management offset by continued investment in Galafold, Pombiliti + Opfolda clinical studies, as well as global launch activities<sup>4</sup>.

## **Amicus is focused on the following key strategic priorities in 2024:**

- Delivering double-digit Galafold revenue growth (11-16% at CER)
- Executing multiple successful launches of Pombiliti + Opfolda
- Advancing ongoing studies to support medical and scientific leadership in Fabry and Pompe diseases
- Achieving full year non-GAAP profitability<sup>2</sup>

<sup>1</sup> In order to illustrate underlying performance, Amicus discusses its results in terms of constant exchange rate (CER) growth. This represents growth calculated as if the exchange rates had remained unchanged from those used in the comparative period. Full-year 2024 Galafold revenue guidance utilizes actual exchange rate as of December 31, 2023.

<sup>2</sup> Based on projections of Amicus' non-GAAP Net (Loss) Income under current operating plans, which includes successful Pombiliti + Opfolda launches and continued Galafold growth. Amicus defines non-GAAP Net (Loss) Income as GAAP Net (Loss) Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, loss on impairment of assets, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, restructuring charges and income taxes.

<sup>3</sup> Full reconciliation of GAAP results to the Company's non-GAAP adjusted measures for all reporting periods appear in the tables to this press release.

<sup>4</sup> A reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure is not available without unreasonable effort due to high variability, complexity, and low visibility as to the items that would be excluded from the GAAP measure.

## **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, February 28, 2024, at 8:30 a.m. ET to discuss the full-year 2023 financial results and corporate updates. Participants and investors interested in accessing the call by phone will need to register using the [online registration form](#). After registering, all phone participants will receive a dial-in number along with a personal PIN number to access the event.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at [ir.amicusrx.com](#). Web participants are encouraged to register on the website 15 minutes prior to the start of the call. An archived webcast and accompanying slides will be available on the Company's website shortly after the conclusion of the live event.

## **About Galafold**

Galafold<sup>®</sup> (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable galactosidase alpha gene (*GLA*) variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of people living with Fabry disease may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in more than 40 countries around the world, including the U.S., EU, U.K., and Japan.



#### U.S. INDICATIONS AND USAGE

Galafold is indicated 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.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

#### U.S. IMPORTANT SAFETY INFORMATION

##### ADVERSE REACTIONS

The most common adverse reactions reported with Galafold ( $\geq 10\%$ ) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

##### USE IN SPECIFIC POPULATIONS

There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus.

It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition.

Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

The safety and effectiveness of Galafold have not been established in pediatric patients.

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.amicusrx.com/pi/Galafold.pdf>.

##### **About Pombiliti + Opfolda**

Pombiliti + Opfolda, is a two-component therapy that consists of cipaglucosidase alfa-atga, a bis-M6P-enriched rhGAA that facilitates high-affinity uptake through the M6P receptor while retaining its capacity for processing into the most active form of the enzyme, and the oral enzyme stabilizer, miglustat, that's designed to reduce loss of enzyme activity in the blood.

#### U.S. INDICATIONS AND USAGE

POMBILITI in combination with OPFOLDA is indicated for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing  $\geq 40$  kg and who are not improving on their current enzyme replacement therapy (ERT).

#### SAFETY INFORMATION

**HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS:** Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, POMBILITI should be discontinued immediately and appropriate medical treatment should be initiated. **INFUSION-ASSOCIATED REACTIONS (IARs):** If severe IARs occur, immediately discontinue POMBILITI and initiate appropriate medical treatment. **RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS:** Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function, may be at risk of serious exacerbation of their cardiac or respiratory status during POMBILITI infusion. See PI for complete Boxed Warning. **CONTRAINDICATION:** POMBILITI in combination with Opfolda is contraindicated in pregnancy. **EMBRYO-FETAL TOXICITY:** May cause embryo-fetal harm. Advise females of reproductive potential of the potential risk to a fetus and to use effective contraception during treatment and for at least 60 days after the last dose. **Adverse Reactions:** Most common adverse reactions  $\geq 5\%$  are headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia. Please see full PRESCRIBING INFORMATION, including BOXED WARNING, for POMBILITI (cipaglucosidase alfa-atga) [LINK](#) and full PRESCRIBING INFORMATION for OPFOLDA (miglustat) [LINK](#).



#### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a pipeline of cutting-edge, first- or best-in-class medicines for rare diseases. For more information please visit the company's website at [www.amicusrx.com](http://www.amicusrx.com), and follow on [X](#) and [LinkedIn](#).

#### **Non-GAAP Financial Measures**

In addition to financial information prepared in accordance with U.S. GAAP, this press release also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We use these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses and profitability on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

#### **Forward Looking Statement**

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities and pricing and reimbursement authorities, are based on current information. Actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that required regulatory inspections may be delayed or not be successful and delay or prevent product approval; the potential that we may not be successful in negotiations with pricing and reimbursement authorities; the potential that we may not be successful in commercializing Galafold and/or Pombiliti and Opfolda in Europe, the UK, the US and other geographies; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, the manufacturing, and commercialization of our products. With respect to statements regarding corporate financial guidance and financial goals and the expected attainment of such goals and projections of the Company's revenue, non-GAAP profitability and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2023 to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

#### **CONTACT:**

##### **Investors:**

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##### **Media:**

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TABLE 1

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

	Years Ended December 31,		
	2023	2022	2021
Net product sales	\$ 399,356	\$ 329,233	\$ 305,514
Cost of goods sold	37,326	38,599	34,466
Gross profit	362,030	290,634	271,048
Operating expenses:			
Research and development	152,381	276,677	272,049
Selling, general, and administrative	275,270	213,041	192,710
Changes in fair value of contingent consideration payable	2,583	1,078	6,514
Loss on impairment of assets	1,134	6,616	—
Depreciation and amortization	7,873	5,342	6,209
Total operating expenses	439,241	502,754	477,482
Loss from operations	(77,211)	(212,120)	(206,434)
Other (expense) income:			
Interest income	7,078	3,024	509
Interest expense	(50,149)	(37,119)	(32,471)
Loss on extinguishment of debt	(13,933)	—	(257)
Other (expense) income	(15,886)	4,176	(2,901)
Loss before income tax	(150,101)	(242,039)	(241,554)
Income tax (expense) benefit	(1,483)	5,471	(8,906)
<b>Net loss attributable to common stockholders</b>	<b>\$ (151,584)</b>	<b>\$ (236,568)</b>	<b>\$ (250,460)</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.51)	\$ (0.82)	\$ (0.92)
Weighted-average common shares outstanding — basic and diluted	295,164,515	289,057,198	271,421,986



TABLE 2

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

	December 31,	
	2023	2022
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 246,994	\$ 148,813
Investments in marketable securities	39,206	144,782
Accounts receivable	87,632	66,196
Inventories	59,696	23,816
Prepaid expenses and other current assets	49,533	40,209
<b>Total current assets</b>	<b>483,061</b>	<b>423,816</b>
Operating lease right-of-use assets, net	26,312	29,534
Property and equipment, less accumulated depreciation of \$25,429 and \$22,281 at December 31, 2023 and 2022, respectively	31,667	30,778
Intangible assets, less accumulated amortization of \$2,510 and \$0 at December 31, 2023 and December 31, 2022, respectively	20,490	23,000
Goodwill	197,797	197,797
Other non-current assets	18,553	19,242
<b>Total Assets</b>	<b>\$ 777,880</b>	<b>\$ 724,167</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 15,120	\$ 15,413
Accrued expenses and other current liabilities	144,245	93,636
Contingent consideration payable	—	21,417
Operating lease liabilities	8,324	8,552
<b>Total current liabilities</b>	<b>167,689</b>	<b>139,018</b>
Long-term debt	387,858	391,990
Operating lease liabilities	48,877	51,578
Other non-current liabilities	13,282	18,534
<b>Total liabilities</b>	<b>617,706</b>	<b>601,120</b>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 293,594,209 and 281,108,273 shares issued and outstanding at December 31, 2023 and 2022, respectively	2,918	2,815
Additional paid-in capital	2,836,018	2,664,744
Accumulated other comprehensive gain (loss):		
Foreign currency translation adjustment	5,429	(11,989)
Unrealized loss on available-for-sale securities	(188)	(116)
Warrants	71	83
Accumulated deficit	(2,684,074)	(2,532,490)
<b>Total stockholders' equity</b>	<b>160,174</b>	<b>123,047</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 777,880</b>	<b>\$ 724,167</b>

TABLE 3

Amicus Therapeutics, Inc.  
Reconciliation of Non-GAAP Financial Measures  
(in thousands)  
(Unaudited)

	Years Ended December 31,		
	2023	2022	2021
<b>Total GAAP operating expenses</b>	\$ 439,241	\$ 502,754	\$ 477,482
<b>Research and development:</b>			
Share-based compensation	21,469	25,089	17,340
<b>Selling, general and administrative:</b>			
Share-based compensation	64,608	51,423	40,498
<b>Loss on impairment of assets</b>	1,134	6,616	—
<b>Changes in fair value of contingent consideration payable</b>	2,583	1,078	6,514
<b>Depreciation and amortization</b>	7,873	5,342	6,209
<b>Total Non-GAAP operating expense adjustments</b>	97,667	89,548	70,561
<b>Total Non-GAAP operating expenses</b>	\$ 341,574	\$ 413,206	\$ 406,921



TABLE 4

**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
(in thousands, except share and per share amounts)  
(Unaudited)

	Three Months Ended December 31,		Years Ended December 31,	
	2023	2022	2023	2022
<b>GAAP net loss</b>	\$ (33,843)	\$ (55,865)	\$ (151,584)	\$ (236,568)
Share-based compensation	18,095	18,626	86,077	76,512
Loss on impairment of assets	—	—	1,134	6,616
Changes in fair value of contingent consideration payable	—	1,584	2,583	1,078
Depreciation and amortization	2,182	1,311	7,873	5,342
Loss on extinguishment of debt	13,933	—	13,933	—
Income tax expense (benefit)	2,183	(14,214)	1,483	(5,471)
Non-GAAP net income (loss)	\$ 2,550	\$ (48,558)	\$ (38,501)	\$ (152,491)
Non-GAAP net income (loss) attributable to common stockholders per common share — basic and diluted	\$ 0.01	\$ (0.17)	\$ (0.13)	\$ (0.53)
Weighted-average common shares outstanding — basic and diluted	300,648,503	289,602,648	295,164,515	289,057,198

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# FY23 Results Conference Call & Webcast

February 28, 2024



# Forward-Looking Statements

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities and pricing and reimbursement authorities, are based on current information. Actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant approval for our product candidates; the potential that required regulatory inspections may be delayed or not be successful and delay or prevent product approval; the potential that we may not be successful in negotiations with pricing and reimbursement authorities; the potential that we may not be successful in commercializing Galafold and Pombiliti and Opfolda in Europe, the UK, the US and other geographies; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, the manufacturing, and commercialization of our products. With respect to statements regarding corporate financial guidance and financial goals and the expected attainment of such goals and projections of the Company's revenue, non-GAAP profitability and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2023 to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*

## Non-GAAP Financial Measures

*In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors a management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. The adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.*

# A Rare Company

A leading biotech company with >\$500M of sales projected in 2024



First Oral Precision Medicine for Fabry Disease

LEVERAGEABLE GLOBAL COMMERCIAL ORGANIZATION



2 APPROVED THERAPIES

World Class Clinical Development Capabilities

Non-GAAP PROFITABILITY Q4 2023 ACHIEVED

>500 EMPLOYEES in 20+ Countries



First Two-Component Therapy for Pompe Disease

\$399M

in 2023 Revenue

21%

Increase Year-Over-Year

Expect Full Year 2024 Non-GAAP Profitability

Combined Peak Revenue Potential \$1.5B – \$2B

# 2024 Strategic Priorities

A Transformative  
Year Ahead for  
Amicus

1

Drive double-digit Galafold<sup>®</sup> revenue growth of 11-16% at C

2

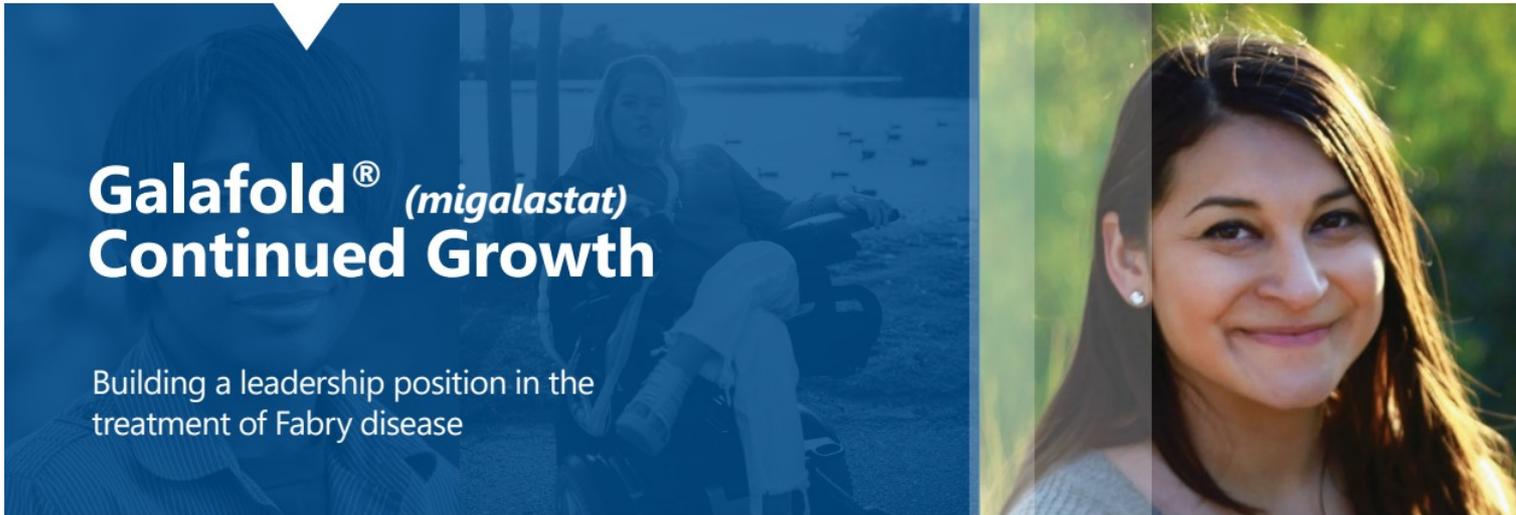
Execute multiple successful launches of Pombiliti<sup>™</sup> + Opfolda

3

Advance ongoing studies to support medical and scientific leadership in Fabry and Pompe diseases

4

Achieve non-GAAP profitability for the full year



# Galafold® (*migalastat*) Continued Growth

Building a leadership position in the  
treatment of Fabry disease

**Galafold is the only approved oral treatment option in Fabry disease**

**A unique mechanism of action for Fabry patients with amenable variants**

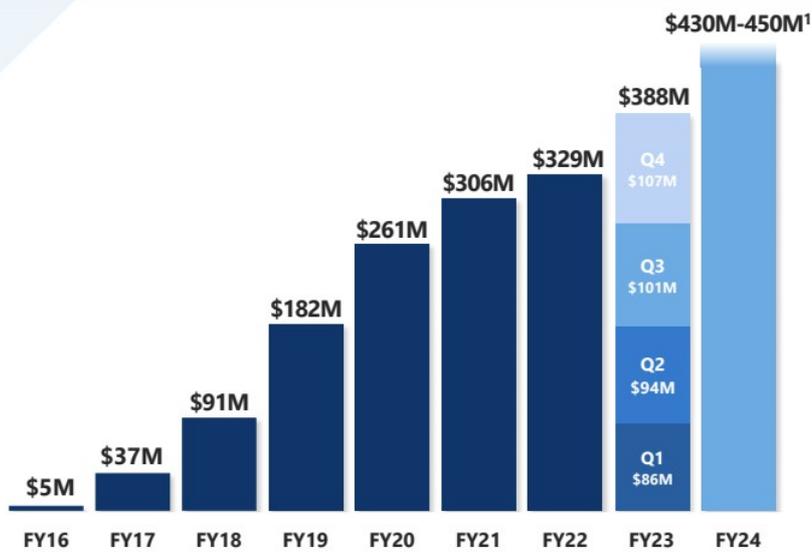


Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.amicusrx.com/pi/Galafold.pdf>. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions, and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).



# Galafold Performance

Galafold YTD reported revenue growth of +18% to \$388M



- Global mix of switch (~43%) and previously untreated patients (~57%)<sup>2</sup>
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

### Distribution of Galafold revenue by quarter over previous 5 years:

	Q1	Q2	Q3	Q4
5 Year Avg.	22%	24%	26%	28%

FY24 revenue growth guidance to 11% to 16% at CER

# Key Growth Drivers for 2024

Building off a strong year with highest patient demand seen in last four years to lay the groundwork for continued double-digit Galafold growth in 2024

- Increasing patient identification through ongoing medical education, screening, and improved diagnostics
- Driving market share of treated amenable patients through excellent execution
- Expanding market through uptake in naïve population as well as geographic and label expansion
- Maintaining >90% adherence and compliance through HCP and patient education and support

**Pombiliti™** (*cipaglucosidase alfa-atga*)  
+  
**Opfolda™** (*miglustat*)

Potential to establish a new standard of care  
for people living with late-onset Pompe disease



# Successful Global Launch of Pombiliti + Opfolda Underway

FY 2023 revenue of \$11.6M (\$8.5M in Q4 2023) provides strong foundation for 2024



## Patient Demand

*As of early January 2024*

~120 patients treated with commercial product or scheduled to be treated

~105 patients from clinical trials and early access

~15 new patients from competitor ERTs or naïve

Very positive early feedback from real-world experience



## KOL Outreach

Successfully engaged with top prescribers in each approved country

Existing relationships with HCPs at key treatment centers

Ongoing disease education



## Access and Reimbursement

Positive interactions with US, UK, and EU payors

Focus on broad patient access

Country-by-country reimbursement process underway

Multiple launches expected in 2H 2024



**Pombiliti™**  
(cipaglucosidase alfa-atga)

+

**Opfolda™**  
(miglustat) 65 mg capsules



Focus in 2024 is on maximizing the number of patients on therapy by year end

# Regulatory and Clinical Updates

## Continuing to build the body of evidence and expand commercial access

- > 10 reimbursement dossiers and multiple regulatory submissions throughout 2024
- Ongoing clinical studies in children with late-onset Pompe disease (LOPD) and infantile-onset Pompe disease (IOPD)
- Amicus registry for Pompe disease to continue generating evidence on differentiated MOA and long-term effect
- Significant presence at *WORLDSymposium™* 2024 with 11 posters and an oral presentation highlighting work in Fabry and Pompe



## Corporate Outlook

Delivering on our mission for patients  
and shareholders

# FY 2023 Select Financial Results

2023 revenue of \$399.4M, up 20% at CER, and net loss significantly reduced

*(in thousands, except per share data)*

	Dec. 31, 2023	Dec. 31, 2022
Product Revenue	\$399,356	\$329,233
Cost of Goods Sold	37,326	38,599
R&D Expense	152,381	276,677
SG&A Expense	275,270	213,041
Changes in Fair Value of Contingent Consideration	2,583	1,078
Loss on Impairment of Assets	1,134	6,616
Depreciation and Amortization	7,873	5,342
Loss from Operations	(77,211)	(212,120)
Interest Income	7,078	3,024
Interest Expense	(50,149)	(37,119)
Loss on Extinguishment of Debt	(13,933)	—
Other (Expense) Income	(15,886)	4,176
Income Tax (Expense) Benefit	(1,483)	5,471
Net Loss	(151,584)	(236,568)
Net Loss Per Share	(0.51)	(0.82)

# Financial Outlook and Path to Profitability

Clear strategy to build our business, advance our portfolio, and achieve profitability



## Sustain Revenue Growth

**\$399M** FY23 revenue,  
**+21%** YoY growth

**>\$500M** in total  
revenue in FY24



## Successfully Launch Pombiliti + Opfolda

Galafold and  
Pombiliti + Opfolda  
expected to drive  
strong **double-digit  
growth long term**



## Deliver on Financial Goals

Focused on disciplined  
expense management

FY24 non-GAAP operating  
expense guidance of  
**\$345M-\$365M**

Achieve FY24  
non-GAAP profitability<sup>1</sup>

# Positioned for Significant Value Creation in 2024

Unlocking the value of two unique commercial therapies in sizeable and growing markets



Accelerating  
total revenue  
growth



Delivering  
full-year  
non-GAAP<sup>1</sup>  
profitability



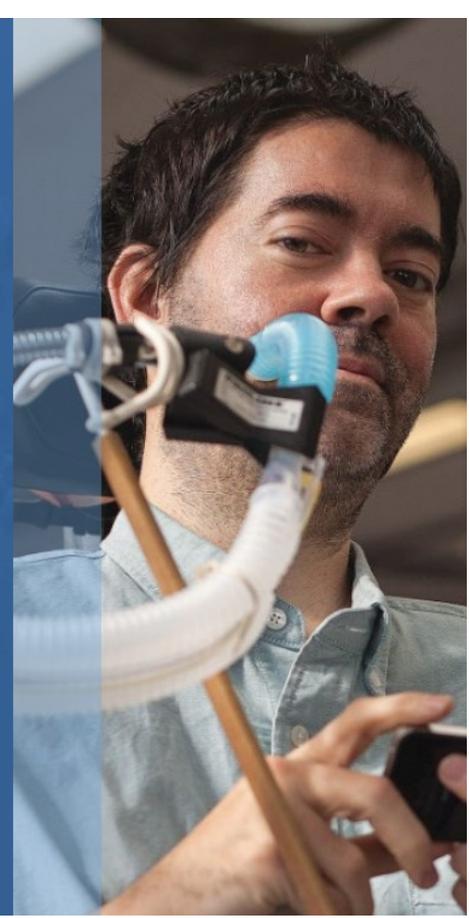
Clear line of  
sight to  
generating  
positive  
cashflow

15 <sup>1</sup> Non-GAAP Net (Loss) Income defined as GAAP Net (Loss) Income excluding the impact of stock-based compensation expense, changes in fair value of contingent consideration, loss on impairment of assets, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, restructuring charges and income taxes.





# Appendix



**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
**(in thousands)**  
**(Unaudited)**

	Years Ended December 31,		
	2023	2022	2021
<b>Total GAAP operating expenses</b>	<b>\$ 439,241</b>	<b>\$ 502,754</b>	<b>\$ 477,482</b>
<b>Research and development:</b>			
Share-based compensation	21,469	25,089	17,340
<b>Selling, general and administrative:</b>			
Share-based compensation	64,608	51,423	40,498
<b>Loss on impairment of assets</b>	1,134	6,616	—
<b>Changes in fair value of contingent consideration payable</b>	2,583	1,078	6,514
<b>Depreciation and amortization</b>	7,873	5,342	6,209
<b>Total Non-GAAP operating expense adjustments</b>	<b>97,667</b>	<b>89,548</b>	<b>70,561</b>
<b>Total Non-GAAP operating expenses</b>	<b>\$ 341,574</b>	<b>\$ 413,206</b>	<b>\$ 406,921</b>

## Appendix II

**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
(in thousands, except share and per share amounts)  
(Unaudited)

	Three Months Ended December 31,		Years Ended December 31,	
	2023	2022	2023	2022
<b>GAAP net loss</b>	<b>\$ (33,843)</b>	<b>\$ (55,865)</b>	<b>\$ (151,584)</b>	<b>\$ (236,568)</b>
Share-based compensation	18,095	18,626	86,077	76,512
Loss on impairment of assets	—	—	1,134	6,616
Changes in fair value of contingent consideration payable	—	1,584	2,583	1,078
Depreciation and amortization	2,182	1,311	7,873	5,342
Loss on extinguishment of debt	13,933	—	13,933	—
Income tax expense (benefit)	2,183	(14,214)	1,483	(5,471)
Non-GAAP net income (loss)	<u>\$ 2,550</u>	<u>\$ (48,558)</u>	<u>\$ (38,501)</u>	<u>\$ (152,491)</u>
Non-GAAP net income (loss) attributable to common stockholders per common share — basic and diluted	\$ 0.01	\$ (0.17)	\$ (0.13)	\$ (0.53)
Weighted-average common shares outstanding — basic and diluted	300,648,503	289,602,648	295,164,515	289,057,198

# Environmental, Social, & Governance (ESG) Snapshot

## Who We Serve

Programs we invest in have 3 key characteristics

- Address a rare genetic disease
- First-in-class or best-in-class
- Impart meaningful benefit for patients



## Pledge for a Cure

Designate a portion of product revenue back into R&D for that specific disease until there is a cure.

## Pricing PROMISE

Committed to never raising the annual price of our products more than consumer inflation.

## Charitable Giving

Contributions allocated:  
**\$2,288,998** U.S.  
**\$954,349** Intl.

Expanded Access through Jan 2024:  
**32** patients / **24** countries

Amicus-supported community programs: **22**  
 Volunteer hours (U.S.): **580**

## Environmental Management

Committed to producing transformative medicines for people living with rare diseases while practicing environmental responsibility and adhering to sustainability best practices in our operations.

*Our mission is to drive sustainability with our partners by incorporating environmental and sustainability principles into all our commercial relationships*

**0%** Amicus-owned Direct Manufacturing and Related GHG Emissions

Global Employees **517**      % Female Employees **58%**

## Board of Directors

Committed to ongoing Board refreshment and diversity of background, gender, skills, and experience:

Director Diversity **80%** Board Independence  
 3 Female  
 2 Veteran Status  
 1 African American  
**60%** Overall Board Diversity

## Diversity, Equity, & Inclusion (DEI)

Pledge to support a more inclusive culture to include our employees, our communities, and society.

Goal of maintaining gender diversity while increasing overall diversity throughout our global workforce.

## Employee Recruitment, Engagement, & Retention

Leverage employee capabilities and expertise to build a culture that drives performance and ultimately attracts, energizes, and retains critical talent.

*Pulse surveys reveal employees feel high personal satisfaction in their job, are proud of their work, and what they contribute to the community*

## Career Development

Reimagined performance management process to measure the *what* and the *how*, rewarding those who role-model our **Mission-focused Behaviors**.



# FX Sensitivity and Galafold Distribution of Quarterly Sales

## Impact from Foreign Currency Q4 2023:

Currency Variances: USD/	Q4 2022	Q4 2023	YoY Variance
EUR	1.021	1.076	5.4%
GBP	1.174	1.241	5.7%
JPY	0.007	0.007	(4.4%)

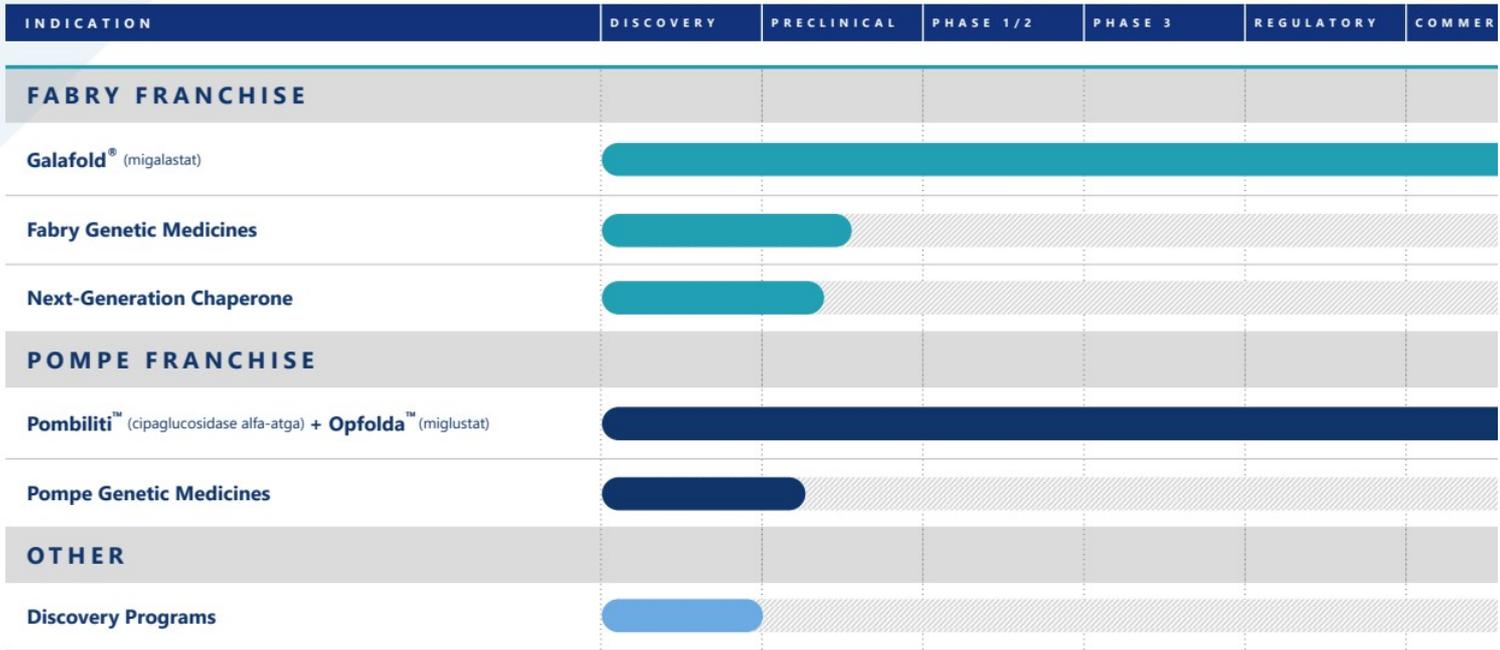
## Distribution of Galafold Revenue by Quarter over Past 5 Years:

	Q1	Q2	Q3	Q4
5 Year Avg.	22%	24%	26%	28%

## Full-year 2024 Revenue Sensitivity

Given the high proportion of Amicus revenue Ex-US (~60%), a change in exchange rates of +/- 5% compared to year-end 2023 rates could lead to a \$15M move in global reported revenues in 2024.

# Streamlined Rare Disease Pipeline with Focus on Fabry Disease and Pompe Disease Franchises





**Thank you**

