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

February 28, 2024 at 7:00 AM EST

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, N.J., Feb. 28, 2024 (GLOBE NEWSWIRE) -- 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 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)[1] 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[1]	2023	2022	Reported	at CER[1]
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 WORLD*Symposium* ™**. 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[3] 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[1] 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[4].

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[2]

[1] 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.

[2] 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.

[3] 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.

[4] 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® (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 (≥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.

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 ≥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 ≥ 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, 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.

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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)
Net loss attributable to common stockholders	$ (151,584)	$ (236,568)	$ (250,460)
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**
Assets		
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
Total current assets	483,061	423,816
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
Total Assets	$ 777,880	$ 724,167
Liabilities and Stockholders' Equity		
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
Total current liabilities	167,689	139,018
Long-term debt	387,858	391,990
Operating lease liabilities	48,877	51,578
Other non-current liabilities	13,282	18,534
Total liabilities	617,706	601,120
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)
Total stockholders' equity	160,174	123,047
Total Liabilities and Stockholders' Equity	$ 777,880	$ 724,167

TABLE 3

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

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