Amicus Therapeutics Announces Full-Year 2022 Financial Results and Corporate Updates

March 1, 2023

2022 Revenue Growth of 16% at CER to $329M

Projecting Galafold Revenue Growth in 2023 of 12-17% at CER

U.S. FDA Pre-approval Inspection for AT-GAA Now Scheduled; Approval expected in 3Q 2023

EU and U.K. AT-GAA Regulatory Reviews On-Track; Approvals expected in 3Q 2023

Non-GAAP Profitability Anticipated in 2H 2023

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

PHILADELPHIA, March 01, 2023 (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, 2022.

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “In 2022, we made significant progress across the business heading into what we believe will be a pivotal year for Amicus. We delivered strong growth in Galafold, highlighted by the over 2,000 people around the world who are now on treatment. We set a strong foundation for the anticipated global launch of our second product. And finally, we streamlined our pipeline while continuing our judicious management of resources. In 2023, we remain laser focused on continuing to grow Galafold globally at double-digit rates, preparing for the expected approvals and launches of AT-GAA for Pompe disease in multiple major markets, and maintaining our financial discipline in order to achieve non-GAAP profitability in the second half of this year. These strategic priorities align with our mission to deliver innovative treatments that impart a meaningful difference in the lives of people living with rare diseases.”

Corporate Highlights:

- **Global revenue in the full-year 2022 was $329.2 million**. Full-year revenue represented a year-over-year increase of 8% from total revenue of $305.5 million in the full-year of 2021. Full-year operational revenue growth measured at constant exchange rates (CER)[1] was 16%.

(in thousands)	Three Months Ended December 31,		Year over Year % Growth		Twelve Months Ended December 31,		Year over Year % Growth	
	2022	2021	As Reported	at CER[1]	2022	2021	As Reported	at CER[1]
Net Product Revenues	$88,096	$82,154	7%	16%	$329,233	$305,514	8%	16%

- **For the full-year 2023, the Company anticipates double-digit Galafold revenue growth of 12-17% at CER[1].** Growth is expected to be driven by continued underlying demand from both switch and treatment-naïve patients, geographic expansion, label extensions, continued diagnosis of new Fabry patients, and commercial execution across all major markets, including the U.S., EU, U.K., and Japan.
- **The U.S. Food and Drug Administration (FDA) pre-approval inspection for AT-GAA is scheduled.** Regulatory approval in the U.S. is expected during the third quarter of 2023, pending a successful inspection.
- **The EU and U.K. AT-GAA regulatory reviews remain on-track with approvals expected in 3Q 2023.** The Committee for Medicinal Products for Human Use (CHMP) previously adopted a positive opinion of Pombiliti™, also known as cipaglucosidase alfa. A CHMP opinion for miglustat, the enzyme stabilizer component of AT-GAA is expected in the second quarter 2023. The regulatory submission process for AT-GAA in the U.K. was initiated in December 2022, with final approval expected in the third quarter of 2023.
- **Expanded access programs continue to meet the growing demand for AT-GAA across multiple countries**. In the U.K., under the Early Access to Medicines Scheme (EAMS), multiple physicians have requested access from each of the leading Pompe centers in the country. Many patients with Pompe disease are participating in additional expanded access programs in the U.S., Germany, France, and Japan.
- **Two oral presentations and 11 posters highlighting Amicus’ development programs in Fabry disease and Pompe disease presented at the 19th Annual WORLD*Symposium*™ 2023.**Updated long-term efficacy and safety data from the global Phase 3 open-label extension study of AT-GAA in late-onset Pompe disease (LOPD) demonstrated consistency and

durability of effect in patients out to two years, suggesting long-term benefit of treatment for people living with LOPD. Initial results from the FollowME Fabry Pathfinders registry was presented showing stable renal function out to 3-years for patients on Galafold.

- **Galafold U.S. intellectual property estate strengthened following the issuance of 19 new patents in 2022.** Galafold is protected by orphan drug regulatory exclusivities and a broad U.S. intellectual property portfolio of 46 orange book-listed patents, including 5 composition of matter patents, 30 of which provide protection through at least 2038.
- **Full-year 2023 non-GAAP operating expense guidance of $340 million to $360 million**, driven by prudent expense management while maintaining AT-GAA manufacturing and pre-launch activities.
- **Based on the current operating plan, the timing of AT-GAA approvals, and through careful management of expenses, the Company is on-track to achieve non-GAAP profitability[2] in the second half of 2023.**

Full-Year 2022 Financial Results

- Total revenue in the full-year 2022 was $329.2 million, a year-over-year increase of 8% from total revenue of $305.5 million in the full-year 2021. On a constant currency basis, full-year 2022 total revenue growth was 16%. Reported revenue was offset by a negative currency impact of $26.1 million, or 8%.
- Cash, cash equivalents, and marketable securities totaled $293.6 million at December 31, 2022, compared to $482.5 million at December 31, 2021.
- Total GAAP operating expenses of $502.8 million for the full-year 2022 increased as compared to $477.5 million for the full-year 2021.
- Total non-GAAP operating expenses of $413.2 million for the full-year 2022 increased slightly as compared to $406.9 million for the full-year 2021, reflecting decreased program spend offset by non-recurring expenses related to the reprioritization of the gene therapy portfolio.[3]
- Net loss was $236.6 million, or $0.82 per share, for the full-year 2022, and was reduced compared to a net loss of $250.5 million, or $0.92 per share, for the full-year 2021.

2023 Financial Guidance

- For the full-year 2023, the Company anticipates total Galafold revenue growth between 12 and 17% 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 2023 is $340 million to $360 million, driven by prudent expense management offset by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, in addition to certain non-recurring costs for manufacturing to support the global launch of AT-GAA[4].
- The Company is on-track to achieve non-GAAP profitability[2] in the second half of 2023.

Amicus is focused on the following five key strategic priorities in 2023:

- Sustain double-digit Galafold revenue growth (12-17% at CER[1])
- Secure FDA, EMA, and MHRA approvals for AT-GAA
- Initiate successful global launches of AT-GAA
- Advance next generation pipeline programs (Fabry GTx, Fabry Next-Generation Chaperone, Pompe GTx)
- Maintain strong financial position on path to profitability

[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 2022 Galafold revenue guidance utilizes the average actual exchange rates for 2021.

[2] Based on projections of Amicus' non-GAAP Net Income under current operating plans, which includes successful AT-GAA regulatory approvals and continued Galafold growth. Amicus defines non-GAAP Net Income as GAAP Net 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, March 1, 2023 at 8:30 a.m. ET to discuss the full-year 2022 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 Fabry patients 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.

EU Important Safety Information

Treatment with Galafold should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. Galafold is not recommended for use in patients with a nonamenable mutation.

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m2). The safety and efficacy of Galafold in children less than 12 years of age have not yet been established. No data are available.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- Galafold 123 mg capsules are not for children (≥12 years) weighing less than 45 kg.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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.

About Fabry Disease

Fabry disease is an inherited lysosomal disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which results from mutations in the GLA gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including heart, kidneys, and skin. Accumulation of GL-3 and progressive deterioration of organ function is believed to lead to the morbidity and mortality of Fabry disease. The symptoms can be severe, differ from person to person, and begin at an early age.

About Pompe Disease

Pompe disease is an inherited lysosomal disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function, to a more slowly progressive, late-onset form primarily affecting skeletal muscle and progressive respiratory involvement. Late-onset Pompe disease can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the skeletal muscles and muscles controlling breathing, that worsens over time.

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 Twitter 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 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.

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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, 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, including the FDA, EMA, and PMDA, 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 commercializing Galafold in Europe, Japan, the US and other geographies or AT-GAA if and when approved; 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 and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue 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, 2022 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,		
	2022	**2021**	**2020**
Net product sales	$ 329,233	$ 305,514	$ 260,886
Cost of goods sold	38,599	34,466	31,044
Gross profit	290,634	271,048	229,842
Operating expenses:			
Research and development	276,677	272,049	308,443
Selling, general, and administrative	213,041	192,710	156,407
Changes in fair value of contingent consideration payable	1,078	6,514	3,144
Loss on impairment of assets	6,616	—	—
Depreciation and amortization	5,342	6,209	8,846
Total operating expenses	502,754	477,482	476,840
Loss from operations	(212,120)	(206,434)	(246,998)
Other income (expense):			
Interest income	3,024	509	3,226
Interest expense	(37,119)	(32,471)	(22,425)
Loss on extinguishment of debt	—	(257)	(7,276)
Other income (expense)	4,176	(2,901)	(781)
Loss before income tax	(242,039)	(241,554)	(274,254)
Income tax benefit (expense)	5,471	(8,906)	(2,598)
Net loss attributable to common stockholders	$ (236,568)	$ (250,460)	$ (276,852)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.82)	$ (0.92)	$ (1.07)
Weighted-average common shares outstanding — basic and diluted	289,057,198	271,421,986	258,867,380

TABLE 2

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

	December 31,	
	2022	**2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 148,813	$ 245,197
Investments in marketable securities	144,782	237,299
Accounts receivable	66,196	52,672
Inventories	23,816	26,818
Prepaid expenses and other current assets	40,209	34,848
Total current assets	423,816	596,834
Operating lease right-of-use assets, net	29,534	20,586
Property and equipment, less accumulated depreciation of $22,281 and $19,882 at December 31, 2022 and 2021, respectively	30,778	42,496
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	19,242	24,427
Total Assets	$ 724,167	$ 905,140
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 15,413	$ 21,513
Accrued expenses and other current liabilities	93,636	98,153
Contingent consideration payable	21,417	18,900
Operating lease liabilities	8,552	7,409
Total current liabilities	139,018	145,975
Long-term debt	391,990	389,357
Operating lease liabilities	51,578	43,363

Deferred income taxes	4,939	4,930
Deferred reimbursements	4,656	5,906
Other non-current liabilities	8,939	8,240
Total liabilities	601,120	597,771
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 281,108,273 and 278,912,800 shares issued and outstanding at December 31, 2022 and 2021, respectively	2,815	2,808
Additional paid-in capital	2,664,744	2,595,419
Accumulated other comprehensive (loss) gain:		
Foreign currency translation adjustment	(11,989)	5,251
Unrealized loss on available-for-sale securities	(116)	(270)
Warrants	83	83
Accumulated deficit	(2,532,490)	(2,295,922)
Total stockholders' equity	123,047	307,369
Total Liabilities and Stockholders' Equity	$ 724,167	$ 905,140

TABLE 3

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

	December 31		
	2022	**2021**	**2020**
Total operating expenses - as reported GAAP	**$ 502,754**	**$ 477,482**	**$ 476,840**
Research and development:			
Share-based compensation	25,089	17,340	20,817
Selling, general and administrative:			
Share-based compensation	51,423	40,498	28,334
Loss on impairment of assets	6,616	—	—
Changes in fair value of contingent consideration payable	1,078	6,514	3,144
Depreciation and amortization	5,342	6,209	8,846
Total operating expense adjustments to reported GAAP	89,548	70,561	61,141
Total operating expenses - as adjusted	$ **413,206**	$ **406,921**	$ **415,699**