



Amicus Therapeutics Reports Preliminary 2022 Revenue and Provides 2023 Strategic Outlook

January 9, 2023

Significant Growth in Demand with More Than 2,000 People Living with Fabry Disease on Galafold by End of 2022

2022 Full Year Revenue of ~\$329M, Representing 16% YoY Growth at CER

Continued Double-Digit Growth in Galafold Revenue of 12-17% at CER Expected in 2023

Multiple Approvals and Launches Expected in 2023 for AT-GAA in Pompe Disease

On-Track to Achieve non-GAAP Profitability in 2H2023

PHILADELPHIA, Jan. 09, 2023 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today provided its preliminary and unaudited 2022 revenue, corporate updates, and full-year 2023 outlook.

Corporate Highlights:

- **Global revenue in 2022 reached \$329 million** (preliminary and unaudited) driven by strong new patient accruals and sustained patient adherence, representing a year-over-year operational revenue growth measured at constant exchange rates (CER)¹ of 16%. Full-year revenue growth measured at actual exchange rates was 8% reflecting a negative currency impact of approximately \$26 million, or 8%, in 2022. Fourth quarter revenue was approximately \$88 million (preliminary and unaudited).
- **For the full-year 2023, the Company anticipates double-digit Galafold revenue growth of 12-17% at CER.** Growth is expected to be driven by continued underlying demand from both switch and treatment-naïve patients, geographic expansion, continued diagnosis of new Fabry patients, and commercial execution across all major markets, including the U.S., EU, U.K., and Japan.
- **Multiple approvals and launches expected in 2023 for AT-GAA in Late-onset Pompe disease.** In Europe, the Committee for Medicinal Products for Human Use (CHMP) 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 second half of 2023. As previously announced, in the U.S., the Food and Drug Administration (FDA) deferred action on AT-GAA. Amicus remains actively engaged with the Agency on developing a plan and logistics for the pre-approval inspection and once there is more clarity, will provide expected approval timing.
- **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., France, Germany, and Japan.
- **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.
- **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 profitability² in the second half of 2023.**

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "In 2022, Amicus remained steadfast in our mission to transform the lives of people living with rare diseases with excellent progress made across our strategic priorities. The Galafold business remained very strong last year, delivering double-digit operational revenue growth and finishing the year with over 2,000 patients on Galafold. We continue to expect robust growth again for 2023 driven by patient demand across the globe for this precision medicine for Fabry disease. In Pompe disease, we eagerly anticipate multiple AT-GAA regulatory approvals in key geographies including the EU, U.K., and U.S. following completion of the FDA inspection, and look forward to launching our second commercial product. We remain excited for the opportunity to offer a new and innovative treatment option, one that we believe has the potential to be the new standard of care, to people living with Pompe disease around the world. We believe we have the opportunity to deliver significant value for our shareholders as Amicus transforms into a leading global rare disease biotechnology company with two innovative therapies that can make a significant impact on the lives of people living with Fabry disease and Pompe disease."

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

- Sustain double-digit Galafold revenue growth (12-17% at CER)
- 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

Mr. Campbell will discuss the Amicus corporate objectives and key milestones in a presentation at the 41st Annual J.P. Morgan Healthcare Conference on Monday, January 9, 2023, at 2:15 p.m. PT. A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate website at <http://ir.amicusrx.com/events.cfm>, and will be archived for 90 days.

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 2023 Galafold revenue guidance utilizes actual exchange rate at December 31, 2022.

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. We define 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, loss on impairment of assets, restructuring charges, and income taxes.

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 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, 2021 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2022. 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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