Amicus Therapeutics Reports Preliminary 2023 Revenue and Provides 2024 Strategic Outlook

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2023 Total Revenue of ~$399.4M, a 21% Increase Year-Over-Year

>2,400 People Living with Fabry Disease on Galafold® Following a Year of Increased Demand

Expecting 2024 Galafold Revenue Growth of 11-16% at CER

Successful Launches of Pombiliti ™ + Opfolda ™ Underway in the U.S., U.K., and Germany

PRINCETON, N.J., Jan. 07, 2024 (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 2023 revenue, corporate updates, and full-year 2024 outlook.

In 2023, Amicus met or exceeded its strategic priorities, highlighted by:

- Sustaining double-digit Galafold revenue growth
- Securing FDA, EMA, and MHRA approvals for Pombiliti + Opfolda
- Initiating successful global launches of Pombiliti + Opfolda
- Advancing next-generation pipeline programs
- On-track to achieving non-GAAP profitability in the fourth quarter of 2023

Preliminary and Unaudited 2023 Revenue:

- **Total revenue in 2023 reached ~$399.4 million**, representing a year-over-year **increase of 21%,** reflecting strong operational growth measured at constant exchange rates (CER)[1] of 20% and a favorable currency impact of approximately $2.7 million, or 1%. Fourth quarter total revenue was ~$115.1 million.
- **Galafold (migalastat) net product sales in 2023 were ~$387.8 million,** representing a year-over-year **increase of 18%, or 17% at CER.** Fourth quarter Galafold net product sales were ~$106.6 million.
- **Pombiliti (cipaglucosidase alfa-atga) + Opfolda (miglustat) net product sales in 2023 were ~$11.6 million.** The commercial launch of Pombiliti + Opfolda is successfully underway in the three largest markets with ~120 patients on treatment with commercial product or scheduled to be treated as of the end of 2023. Fourth quarter Pombiliti + Opfolda net product sales were ~$8.5 million.

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “Last year was an incredible year for Amicus highlighted by the continued double-digit growth of Galafold sales and the global regulatory approvals of our second commercial therapy, which is off to a fantastic launch. We expect 2024 to be a truly transformative year as we continue to drive significant revenue growth by treating an increasing number of Fabry patients globally, executing on the global launches of Pombiliti and Opfolda, which we believe has the potential to become the standard of care in a >$1B market today, and delivering our first full year of non-GAAP profitability. We look forward to reporting on our progress throughout this year as we further our mission for people living with rare diseases.”

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

- Delivering double-digit Galafold revenue growth (11-16% at CER)
- Ensuring the successful global 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]

Mr. Campbell will discuss the Amicus corporate objectives and key milestones in a presentation at the 42nd Annual J.P. Morgan Healthcare Conference on Monday, January 8, 2024, 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 2024 Galafold revenue guidance utilizes actual exchange rate as of December 31, 2023.

[2] Non-GAAP Net Income defined 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 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 drug reactions reported with Galafold (≥10 %) are headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia.

DRUG INTERACTIONS

Avoid co-administration of Galafold with caffeine at least 2 hours before and 2 hours after taking Galafold.

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 Therapeutic Indication

Galafold® (migalastat) is indicated for long-term treatment of adults and adolescents aged 12 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation.

EU Important Safety Information

Treatment with Galafold should be initiated and supervised by specialist physicians experienced in the diagnosis and treatment of Fabry disease. Galafold is not intended for concomitant use with enzyme replacement therapy.

The safety and efficacy of Galafold in children aged less than 12 years have not been established. No data are available.

Galafold is contraindicated in patients with hypersensitivity to the active substance or to any of the excipients listed in the Summary of Product Characteristics (SmPC).

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 or switched to Galafold. In case of meaningful clinical deterioration, further clinical evaluation or discontinuation of treatment with Galafold should be considered.

Galafold is not indicated for use in patients with non-amenable mutations.

Galafold is not recommended for use in patients with severe renal insufficiency, defined as estimated GRF less than 30 mL/min/1.73m^2.

Food and caffeine should not be consumed at least 2 hours before and 2 hours after taking Galafold to give a minimum 4 hours fast.

Galafold is not recommended in women of childbearing potential not using contraception. Galafold is not recommended during pregnancy. It is not known whether Galafold is secreted in human milk.

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 SmPC.

OVERDOSE: General medical care is recommended in the case of Galafold overdose.

For complete information please see the EU SmPC available at https://www.ema.europa.eu/en/medicines/human/EPAR/galafold

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.**

EU Important Safety Information
Pombiliti (cipaglucosidase alfa) Important Safety Information

Posology and Method of Administration: Pombiliti must be used in combination with miglustat 65 mg hard capsules. The recommended dose of Pombiliti is 20 mg/kg of body weight every other week. The Pombiliti infusion should start 1 hour after taking miglustat capsules. ***Paediatric population:*** The safety and efficacy of Pombiliti in combination with miglustat therapy in paediatric patients less than 18 years old have not yet been established. No data are available. ***Contraindications*****:** Life-threatening hypersensitivity to the active substance, or to any of the excipients. Contraindication to miglustat. ***Anaphylaxis and infusion-associated reactions (IARs):*** Serious anaphylaxis and IARs have occurred in some patients during infusion and following infusion with Pombiliti. Premedication with oral antihistamine, antipyretics, and/or corticosteroids may be administered to assist with signs and symptoms related to IARs experienced with prior enzyme replacement therapy (ERT) treatment. Reduction of the infusion rate, temporary interruption of the infusion, symptomatic treatment with oral antihistamine, or antipyretics, and appropriate resuscitation measures should be considered to manage serious IARs. If anaphylaxis or severe allergic reactions occur, infusion should be immediately paused, and appropriate medical treatment should be initiated. The current medical standards for emergency treatment of anaphylactic reactions are to be observed and cardiopulmonary resuscitation equipment should be readily available. The risks and benefits of re-administering Pombiliti following anaphylaxis or severe allergic reaction should be carefully considered, and appropriate resuscitation measures made available. ***Risk of acute cardiorespiratory failure in susceptible patients:*** Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk of serious exacerbation of their cardiac or respiratory compromise during infusions. Appropriate medical support and monitoring measures should be readily available during Pombiliti infusion. ***Immune complex-related reactions:*** Immune complex-related reactions have been reported with other ERTs in patients who had high IgG antibody titres, including severe cutaneous reactions and nephrotic syndrome. If immune complex-related reactions occur, discontinuation of the administration of Pombiliti should be considered and appropriate medical treatment should be initiated. The risks and benefits of re-administering Pombiliti following an immune complex-related reaction should be reconsidered for each individual patient. ***Contraception in females:*** Reliable contraceptive measures must be used by women of childbearing potential during treatment with Pombiliti in combination with miglustat, and for 4 weeks after discontinuing treatment. ***Pregnancy:*** Pombiliti in combination with miglustat therapy is not recommended during pregnancy. ***Breast feeding:*** It is not known if Pombiliti and miglustat are secreted in human breast milk. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from Pombiliti in combination with miglustat therapy, taking into account the benefit of breast-feeding for the child and the benefit of therapy for the woman. ***Summary of the safety profile:*** The most commonly reported adverse reactions only attributable to Pombiliti were chills (4.0%), dizziness (2.6%), flushing (2.0%), somnolence (2.0%), chest discomfort (1.3%), cough, (1.3%), infusion site swelling (1.3%), and pain (1.3%). Reported serious adverse reactions only attributable to Pombiliti were urticaria (2.0%), anaphylaxis (1.3%), pyrexia (0.7%), presyncope (0.7%), dyspnoea (0.7%), pharyngeal oedema (0.7%), wheezing (0.7%), and hypotension (0.7%). Refer to SmPC for full list.

Opfolda (miglustat) 65 mg hard capsules Important Safety Information

Posology and Method of Administration: Opfolda must be used in combination with Pombiliti. The recommended dose is to be taken orally every other week and is based on body weight. Opfolda should be taken approximately 1 hour but no more than 3 hours before the start of the Pombiliti infusion. **Paediatric population:** The safety and efficacy of Opfolda in combination with Pombiliti therapy in paediatric patients less than 18 years old have not yet been established. No data are available. **Contraindications:** Hypersensitivity to the active substance or to any of the excipients. Contraindication to cipaglucosidase alfa. **Food Interaction:** Patients should fast for 2 hours before and 2 hours after taking Opfolda. **Contraception in females:** Reliable contraceptive measures must be used by women of childbearing potential during treatment with Opfolda in combination with Pombiliti, and for 4 weeks after discontinuing treatment. **Pregnancy:** Opfolda crosses the placenta. Opfolda in combination with Pombiliti therapy is not recommended during pregnancy. **Breast feeding:** It is not known if Opfolda and Pombiliti are secreted in human breast milk. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from Opfolda in combination with Pombiliti therapy, taking into account the benefit of breastfeeding for the child and the benefit of therapy for the woman. **Summary of the safety profile:** The most commonly reported adverse reaction only attributable to Opfolda 65 mg was constipation (1.3%). Refer to SmPC for full list.

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 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 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, 2022, and on Form 10-Q for the quarter ended September 30, 2023. 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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