Amicus Therapeutics Reports Preliminary 2021 Revenue and Provides 2022 Strategic Outlook and Revenue Guidance

January 10, 2022

Full-Year 2021 Galafold® Revenue of ~$306M, Representing 17% YoY Growth

Expect Double-Digit Growth (15-20%) of 2022 Galafold Revenue with $350M-$365M in Global Sales

U.S. and EU Regulatory Reviews Underway for AT-GAA in Pompe Disease

AT-GAA Global Launch Preparations Accelerating

Cash Flow and Balance Sheet Sufficient to Achieve Self-Sustainability and Profitability by 2023

PHILADELPHIA, Jan. 10, 2022 (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 2021 revenue, corporate updates, and full-year 2022 outlook and revenue guidance.

Corporate Highlights:

- **Global revenue for Galafold® (migalastat) in 2021 reached $306 million driven by strong new patient accruals and sustained patient adherence, representing a year-over-year increase of 17%.**
- **AT-GAA regulatory reviews are underway:** In the U.S., the Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA. The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA. In the EU, the Marketing Authorization Applications (MAA) were submitted and validated in the fourth quarter by the European Medicines Agency (EMA).
- **AT-GAA launch preparations are accelerating:** Development of global launch plans, targeted investments in additional personnel, and launch inventory are fully underway as company believes AT-GAA can rapidly become the new standard of care treatment regimen for people living with Pompe disease.
- **Pipeline of next generation genetic medicines to advance through both internal efforts and creation of R&D focused new company, Caritas Therapeutics.**
- **Cash Flow and Balance Sheet sufficient to achieve self-sustainability and profitability in 2023.** Through careful management of expenses, the Company is on the path to achieve self-sustainability and profitability in 2023 as it executes on the global Galafold expansion and prepares for AT-GAA global launch.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “In 2021, Amicus made great strides for people worldwide living with rare diseases through the broad execution of our annual strategic priorities. Despite the resurgence of COVID with Delta and Omicron variants, the Galafold business remains very strong, and we delivered on our full year revenue guidance and expect robust growth this year driven by strong adoption across the globe for our Fabry disease precision medicine. We are underway with the global regulatory reviews and launch preparations for AT-GAA in Pompe disease with high expectations that this novel medicine has the potential to become the new standard of care in Pompe disease treatment and the potential to address unmet needs for thousands of Pompe patients in the years ahead. We see further opportunity ahead to impact the lives of those living with rare disease through our genetic medicine business and capabilities. Together, Amicus is in a stronger position than ever and we remain steadfast on our mission of transforming the lives of people living with rare, life-threatening conditions and creating significant value for our shareholders.”

Bradley Campbell, President and Chief Operating Officer of Amicus Therapeutics, Inc., stated, “We are looking ahead to transforming Amicus into a leading global rare disease biotechnology company led by two innovative therapies that we believe meaningfully impact the lives of people living with Fabry and Pompe disease. This year we will be focused on continuing to bring Galafold to patients around the world and delivering on the anticipated approval and launch of AT-GAA.”

Amicus is focused on the following five key strategic priorities in 2022:

- Continued double-digit Galafold growth (15-20%) with revenue of $350M to $365M
- Secure FDA approval and positive CHMP opinion for AT-GAA
- Initiate successful, rapid launch in the U.S. for AT-GAA

- Advance best-in-class next generation genetic medicines and capabilities
- Maintain strong financial position on path to profitability

Mr. Crowley and Mr. Campbell will discuss the Amicus corporate objectives and key milestones in a presentation at the 40th Annual J.P. Morgan Healthcare Conference on Wednesday, January 12, 2022, at 3:45 p.m. ET. 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.

Full-Year 2021 Revenue Summary and 2022 Revenue Guidance

Global revenue for Galafold in full-year 2021 was approximately $306 million, preliminary and unaudited, representing a year-over-year increase of 17% from total revenue of $260.9 million in 2020. Full-year revenue benefited from a positive currency impact of approximately $7 million. Fourth quarter Galafold revenue was approximately $84 million, preliminary and unaudited.

For the full-year 2022, the Company anticipates total Galafold revenue of $350 million to $365 million. Double-digit revenue growth (15-20%) in 2022 is expected to be driven by continued underlying demand from both switch and naïve patients, geographic expansion, the continued diagnosis of new Fabry patients and commercial execution across all major markets, including the U.S., EU, U.K., and Japan.

The current cash position is sufficient to achieve self-sustainability and profitability in 2023.

Updates and Anticipated Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Sustain double-digit revenue growth in 2022 of $350 million to $365 million
- Continue geographic expansion
- Registry and other Phase 4 studies ongoing

AT-GAA for Pompe Disease

- U.S. Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA
- EU Committee for Medicinal Products for Human Use (CHMP) opinion expected in late 2022
- Continue to broaden access through early access plans in the U.K., Germany, Japan, and other countries
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Pipeline

- Advance IND-enabling studies, manufacturing activities, and regulatory activities for the Fabry disease gene therapy program towards an anticipated IND in 2023
- Progress preclinical studies, manufacturing activities, and regulatory activities for the Pompe disease gene therapy program
- Discontinue CLN6 Batten disease gene therapy program following review of long-term extension study data. It was recently determined that any initial stabilization of disease progression at the two-year time point was not maintained through the long-term extension study. Amicus plans to further analyze and share the Phase 1/2 data with key stakeholders in the CLN6 Batten disease community and work with the community to support continued research efforts to find better treatments and cures which are so desperately and urgently needed
- Advance CLN3 Batten disease program with the higher dose, different promoter, and intra-cisterna magna (ICM) route of delivery pending further Phase 1/2 clinical data and pre-clinical data expected in 2022. These data will inform timeline for commencement of any pivotal clinical study

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 over 40 countries around the world, including the U.S., EU, U.K., Japan and others.

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 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 metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at www.amicusrx.com, and follow us on Twitter and LinkedIn.

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 in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, 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 our other product candidates 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, 2020 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2021. 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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