Amicus Therapeutics Reports Preliminary 2020 Revenue and Provides 2021 Outlook

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Full-Year 2020 Galafold® Revenue of ~$261M Exceeds Guidance

Strengthened Galafold IP Portfolio Now Includes 24 Issued Patents Including 13 Patents through 2038

Pompe Phase 3 PROPEL Study Last Patient, Last Visit Complete with Data Expected in 1Q2021

AT-GAA Pompe Clinical and CMC Milestones On-Track to Support 2021 BLA and MAA

Multiple Data and Manufacturing Updates and Advancements Planned Across Industry-Leading Rare Disease Gene Therapy Portfolio

Cash Position Sufficient to Achieve Self-Sustainability

CRANBURY, N.J., Jan. 11, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today provided preliminary, unaudited Galafold revenue for the full-year 2020 and introduced its full-year 2021 strategic outlook and financial guidance.

Over the previous year, Amicus substantially met or exceeded its strategic priorities, highlighted by:

- $261 million of global product revenue for Galafold driven by strong adoption and patient adherence
- Completed last patient, last visit of the AT-GAA Phase 3 PROPEL clinical study, advanced manufacturing activities, and initiated the rolling Biologics License Application (BLA) with the U.S. Food and Drug Administration (FDA)
- Advanced clinical development and manufacturing for CLN6 and CLN3 Batten programs
- Progressed Pompe gene therapy and disclosed Fabry as the next gene therapy IND candidate
- Maintained strong financial position

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “During 2020, Amicus remained steadfast on our journey to becoming a leading global rare disease biotechnology company. Despite the extraordinary challenges of COVID, Amicus emerged from 2020 a better and stronger company organizationally, strategically, scientifically and financially. Following continued momentum and strong adoption across the globe for our Fabry precision medicine Galafold, we have again for 2020 exceeded our annual revenue guidance. We are eagerly looking ahead to our Phase 3 readout of AT-GAA in Pompe disease this quarter with high expectations that this novel medicine has the potential to become the new standard of care in Pompe disease treatment. And finally, our world leading gene therapy pipeline gives us tremendous promise in the ability to develop next-generation gene therapies to treat many devastating rare diseases. Amicus is in a stronger position than ever and remains focused on transforming the lives of people living with these rare, life-threatening conditions and creating significant value for our shareholders.”

Amicus is focused on the following five key strategic priorities in 2021:

- Achieve continued double-digit growth and Galafold revenue of at least $300 million+[1]
- Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals
- Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline
- Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs
- Maintain strong financial position

[1] Guidance range to be provided on full-year earnings call.

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 39th Annual J.P. Morgan Healthcare Conference on Tuesday, January 12, 2021, at 8:20 a.m. ET. A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 90 days.

Full-Year 2020 Galafold Summary and 2021 Guidance

Global revenue for Galafold in full-year 2020 was approximately $261 million, preliminary and unaudited, representing a year-over-year increase of 43% from total revenue of $182 million in 2019, and exceeded the Company’s 2020 guidance of $250 million to $260 million despite worsening of the COVID-19 pandemic towards the end of the year. Full-year revenue benefited from a positive currency impact of approximately $2 million. Fourth quarter Galafold revenue was approximately $70 million, preliminary and unaudited. While we observed increased lag times between patient identification and Galafold initiation due to the resurgence of COVID in the fourth quarter, demand for Galafold for Fabry patients with amenable variants worldwide remained strong with queues of potential new Galafold patients in multiple geographies. We also continue to see 90%+ compliance rates among already treated Galafold patients.

For the full-year 2021, the Company anticipates total Galafold of revenue at least $300 million+. Double-digit revenue growth in 2021 is expected to be driven by continued operational growth and commercial execution across all major markets, including the U.S., EU, U.K. and Japan. Non-GAAP

operating expense guidance in 2021 is expected to remain flat at $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies and advancing the gene therapy pipeline. The current cash position is sufficient to achieve self-sustainability without the need for future dilutive financing.

Updates and Anticipated 2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for People Living with Fabry Disease and have an Amenable Variant

- Continued revenue growth in 2021 of at least $300 million+. Guidance range to be provided on full-year earnings call.
- Following the issuance of 11 new patents covering a range of treatment methods, Galafold has 24 issued patents, 13 of which extend IP protection into 2038
- Plans to expand EU label to cover adolescent population
- Continued geographic expansion
- Registry and other Phase 4 studies

AT-GAA For Pompe Disease

- Report data from the AT-GAA Phase 3 PROPEL study in 1Q2021
- Complete the BLA submission in 1H2021 and the EU MAA submission to be completed in 2H2021
- Ongoing supportive studies, including an open-label study in 12- to <18-year-olds living with Pompe and plans to initiate additional pediatric studies in 2021

Gene Therapy Pipeline

- As part of the J.P. Morgan Conference virtual presentation, Amicus will highlight initial preclinical proof of concept data in CLN1 Batten disease and its plans on initiating an early-stage gene therapy program in Angelman Syndrome
- Advance manufacturing and regulatory discussions to finalize clinical and regulatory path for the CLN6 Batten disease gene therapy program and begin dosing additional patients with GMP grade material
- Report initial data from the CLN3 Batten disease Phase 1/2 study, advance manufacturing and regulatory discussions to finalize clinical and regulatory path, and submit IND for next clinical study
- Continue IND-enabling work in both Pompe and Fabry
- Additional preclinical data and potential IND candidate declarations across multiple preclinical programs
- Manufacturing advancements and updates across the portfolio

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 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 m^2). The safety and efficacy of Galafold in children 0–15 years of age have not yet been established.
- 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.
- 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, 2019 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2020. 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.

Non-GAAP Financial Measures

In addition to financial information prepared in accordance with U.S. GAAP, this presentation 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.

CONTACT:

Investors:
Amicus Therapeutics
Andrew Faughnan
Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Amicus Therapeutics
Diana Moore
Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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