Amicus Therapeutics Provides Full-Year 2020 Strategic Outlook and Financial Guidance

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2020 Galafold Revenue Guidance of $250M-$260M

Focused on Pompe Phase 3 PROPEL study and manufacturing to support 2021 BLA and MAA

Continued Progress Across Industry Leading Rare Disease Gene Therapy Portfolio

Strong Balance Sheet with $450M+ Cash – Cash Runway Well into 2022

CRANBURY, N.J., Jan. 13, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global, patient centric biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today provided its full-year 2020 strategic outlook and financial guidance.

Over the course of 2019, Amicus substantially met or exceeded its five key strategic priorities:

- Nearly doubled annual revenue for Galafold with 1,000+ Fabry patients on Galafold by year end
- Completed enrollment in pivotal study in Pompe disease and reported additional Phase 2 data
- Reported additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and substantially completed enrollment in ongoing CLN-3 Batten disease Phase 1/2 study
- Established preclinical proof of concept for Fabry and Pompe gene therapies
- Maintained a strong financial position

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “During 2019, Amicus continued our journey of becoming a leading global rare disease company. Following continued momentum and strong adoption across the globe for our Fabry precision medicine Galafold, we have exceeded our 2019 guidance. Patients are also now being treated in multiple Amicus clinical studies, including our Phase 1/2 and Phase 3 study for AT-GAA in Pompe disease, as well as Phase 1/2 studies of our investigational gene therapies for CLN3 and CLN6 Batten disease. Our first clinical data from our gene therapy pipeline suggested that our CLN6 gene therapy has the potential to stabilize progression in this devastating childhood disease. In addition, our preclinical gene therapy work with our partners at the University of Pennsylvania gives us tremendous promise in the ability to develop next generation gene therapies for the many of patients living with a rare disease. 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 2020:

- Achieve $250 million to $260 million of global product revenue for Galafold
- Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA
- Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs
- Progress Pompe gene therapy towards IND and disclose up to two additional IND candidates
- Maintain strong financial position

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 38th Annual J.P. Morgan Healthcare Conference on Tuesday, January 14, 2020 at 8:30 a.m. PT (11:30 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 2019 Financial Summary and 2020 Guidance

Amicus previously announced full-year 2019 revenue of approximately $181 million (preliminary and unaudited) from commercial sales of Galafold.

For the full-year 2020, the Company anticipates total Galafold revenue of $250 million to $260 million based on the average exchange rates for 2019. Growth in 2020 is expected to be driven by continued growth in EU markets, further success from launches in the U.S. and Japan, as well as reimbursement in additional markets. Non-GAAP operating expense guidance for the year is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies, and advancing our gene therapy pipeline.

Cash, cash equivalents, and marketable securities totaled over $450 million (preliminary and unaudited) at December 31, 2019. The current cash position is anticipated to fund ongoing operations well into 2022.

Program Highlights

Galafold (Migalastat) Oral Precision Medicine for Fabry Disease
Galafold is an oral precision medicine for the treatment of Fabry disease in adults who have amenable *GLA* variants and is approved in over 40 countries around the world, including the U.S., EU, Japan and others.

Global Galafold Updates:

- Full-year 2019 Galafold® revenue of ~$181 million exceeds guidance
- Recent marketing authorizations received in key countries, including Brazil, Columbia and Taiwan
- Registry and other Phase 4 supportive studies underway

AT-GAA for Pompe Disease
AT-GAA is an investigational therapy in Phase 3 development that consists of cipaglucosidase alfa (ATB200), a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose 6-phosphate (M6P), to enhance uptake into cells, co-administered with miglustat (AT2221), a pharmacological chaperone. Positive results from a global Phase 1/2 clinical study (ATB200-02) have shown consistent and durable responses across key measures of safety, functional outcomes and biomarkers in both ERT-switch and ERT-naïve Pompe patients following up to 24 months of treatment with AT-GAA.

Pompe Program Updates:

- Completed enrollment of 123 patients in Phase 3 PROPEL study in 4Q2019 – data expected 1H2021
- Promising Innovative Medicine (PIM) designation issued by MHRA with potential for early access for Pompe patients in United Kingdom based on Phase 1/2 results
- Biologics Manufacturing with WuXi Biologics on Track with PPQ Runs at Commercial Scale

Anticipated Pompe Program Milestones in 2020:

- Plans to apply for and initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020 with addition of full clinical results in 1H2021 to support full approval under Fast Track Designation
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients
- Additional supportive studies, including an open-label study in pediatric patients

Gene Therapy Portfolio
Amicus has established an industry leading gene therapy portfolio of potential therapies for people living with rare diseases, through a license with Nationwide Children's Hospital and an expanded collaboration with the University of Pennsylvania. Our pipeline includes gene therapy programs in rare, neurologic lysosomal disorders, specifically: CLN6, CLN3, CLN8 and CLN1 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder, Niemann-Pick Type C, Mucopolysaccharidosis Type IIIB, as well as a next generation program in Mucopolysaccharidosis Type IIIA. The expanded collaboration with Penn also provides us with exclusive disease-specific access and the option rights to develop potentially disruptive new gene therapy platform technologies and programs for most lysosomal disorders and a broader portfolio of rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy, and select other muscular dystrophies.

Gene Therapy Program Updates:

- In CLN6, received Orphan Designation in the U.S. and EU; Rare Pediatric Disease Designation granted
- In CLN3, received Orphan Designation in the U.S. and EU; Rare Pediatric Disease Designation granted

Anticipated Gene Therapy Pipeline Milestones in 2020:

- Dose additional patients in CLN6 Phase 1/2 study and plan to advance regulatory discussions to finalize clinical and regulatory path
- Initiate long-term follow-up of initial CLN6 patients in Phase 1/2 study in 1H2020 to obtain long-term safety and efficacy data
- Plan to advance regulatory discussions to finalize clinical and regulatory path in CLN3
- Report initial data on patients enrolled in CLN3 Phase 1/2 study
- Complete IND enabling toxicology work in Pompe disease and progress towards IND
- Additional preclinical data expected in multiple programs
- Disclose up to two additional IND candidates
- Manufacturing advancements across 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, 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, business development plans and the projected revenues, sales, expenses 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 or projections 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, 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. With respect to statements regarding projections of the Company's revenue, sales, expenses and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans and strategies. 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, 2018. 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 press 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.

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