U.S. FDA Files New Drug Application Under Priority Review for Migalastat for Treatment of Fabry Disease

February 12, 2018

Six-Month PDUFA Goal Date is August 13, 2018

CRANBURY, N.J., Feb. 12, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced that the U.S. Food and Drug Administration (FDA) has accepted the New Drug Application (NDA) for filing under priority review for the oral precision medicine migalastat HCl ("migalastat") for the treatment of patients 16 years and older with Fabry disease who have amenable mutations. The Prescription Drug User Fee Act (PDUFA) goal date for the FDA decision is August 13, 2018.

Migalastat previously received both Orphan Drug Designation and Fast Track designation from the U.S. FDA. The FDA's Priority Review status accelerates the review time from 10 months to a goal of six months from the day of acceptance of filing and is given to drugs that may offer major advances in treatment or may provide a treatment where no adequate therapy exists.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "The FDA's acceptance of our first Amicus NDA submission under priority review is an important step toward a potential oral precision medicine option for the Fabry disease community in the U.S. With more than a decade of experience in treating patients with migalastat globally, our team at Amicus has collaborated with leading Fabry disease experts and patient organization leaders to assemble a robust NDA that emphasizes the breadth of our clinical data and experience delivering migalastat to patients. We look forward to continuing to work collaboratively with FDA to bring this oral precision medicine to patients who may be able to benefit."

The NDA submission for migalastat is based on clinical data from completed studies, including reduction in disease-causing substrate (GL-3), as well as the totality of data from two Phase 3 pivotal studies in treatment-naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT).

An estimated 3,000 people in the U.S. are currently diagnosed with Fabry disease, more than any other country. Fabry disease is a progressive, inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the *GLA* gene. The disease causes accumulation of specific lipids, primarily GL-3, in tissues including the heart, kidneys, central nervous system, and skin. This abnormal accumulation can lead to debilitating consequences including pain, kidney failure, heart disease, and stroke.

The European Commission (EC) granted full approval for migalastat, under the trade name Galafold™, as a first line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-Gal A deficiency) and who have an amenable mutation. The EC approval was based on clinical data from two Phase 3 pivotal studies (FACETS and ATTRACT), as well as ongoing long-term extension studies.

Outside the EU, migalastat is approved in Switzerland, Israel, Australia, South Korea, and Canada, with regulatory submissions under review in the U.S., Japan, and Taiwan.

About Migalastat and Amenable Mutations

Migalastat is a first-in-class chaperone therapy approved in the European Union as a monotherapy for Fabry disease in patients with amenable mutations. Migalastat works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) has been used to classify more than 1,000 known *GLA* mutations as "amenable" or "not amenable" to treatment with migalastat. The EU label includes 348 *GLA* mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay, which represent between 35% and 50% of the currently diagnosed Fabry population.

Healthcare providers in the EU may access the website www.Galafoldamenabilitytable.com to quickly and accurately identify which mutations are categorized as "amenable" or "not amenable" to migalastat. Amicus expects to submit additional updates to the EU label as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

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 non-amenable 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 storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of 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 Gb_3). 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 the central nervous system, heart, kidneys, and skin. Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease, and stroke. The symptoms can be severe, differ from patient to patient, and begin at an early age. All Fabry disease is progressive and may lead to organ damage regardless of the time of symptom onset.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The lead program in the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the clinical development, regulatory approval pathway, and prospects and timing of regulatory submission and approval of our product candidates for the treatment of Fabry disease. Any express or implied statements contained in this press release that are not statements of historical fact, including interpretation of guidance given by the U.S. FDA, may be deemed forward-looking statements. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved in a timely manner or at all. 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, 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, changes in FDA guidance for regulatory approval, risks regarding the FDA's interpretation of our clinical trial results, including the risk that results from completed clinical trials that supported approval by regulators in other jurisdictions will not be sufficient for U.S. FDA purposes, the risk that the FDA will require additional studies or data, the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidate and the potential that we may not be successful in commercializing our product candidates for Fabry disease in Europe or any other country in which approval is ultimately obtained, if any. 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, 2016 and the Quarterly Report for the quarter ended September 30, 2017. The FDA guidance described in this release was given as of a specific date and the FDA could change its position on the clinical end points or other standards for review and/or approval. 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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Source: Amicus Therapeutics, Inc.