August 15, 2017

Amicus Therapeutics Announces Approval for Galafold™ (Migalastat) for Treatment of Fabry Disease in Australia

First Amicus Medicine and First Oral Precision Medicine for Fabry Disease in Australia

Broad Label for Fabry Patients with an Amenable Genetic Mutation

CRANBURY, N.J., Aug. 15, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) announced that the Australian Therapeutic Goods Administration (TGA) has approved the oral precision medicine Galafold for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. Amicus estimates that approximately 35%-50% of Fabry patients in Australia have an amenable mutation. Following the TGA approval, Amicus is continuing to work with the Australian reimbursement authorities to make Galafold available to Australian patients in a timely manner.

"The approval of Galafold in Australia is a significant step forward for the Fabry community and reflects our commitment to providing the first oral precision medicine for Fabry disease as rapidly as possible to patients throughout the world," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "There has been a tremendous amount of momentum for the commercial launch and significant progress with our regulatory submissions and approvals for Galafold. Following our initial EU approval, this is our second approval through an independent submission process. We have also secured approvals in geographies such as Switzerland and Israel that have a regulatory pathway that accepts the EU approval as the basis for submission and review. Our next step in Australia is to navigate the pricing and reimbursement discussions as we continue toward our vision to deliver Galafold to even more patients in more geographies."

The Australian TGA approval under the Orphan Drug program was based on clinical data from two Phase 3 pivotal studies in both treatment naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT), as well as an ongoing long-term extension study. Fabry disease is a rare genetic disease and potentially life-threatening condition caused by the accumulation of disease substrate (globotriaosylceramide, GL-3) in the lysosome due to a dysfunctional or deficient enzyme. Galafold works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. An amenable mutation is one that is responsive to therapy with Galafold based on a proprietary *in vitro* assay (Galafold Amenability Assay).

"The Australian approval of Galafold paves the way for the first new Fabry treatment option in more than a decade, and provides a clearly differentiated oral precision medicine option for Fabry patients in Australia who have amenable mutations," said Megan Fookes, Managing Director of Fabry Australia. "We are grateful for Amicus' commitment to innovation, patient-focused drug development, and high quality therapies for the Fabry community."

The European Commission granted full approval for Galafold in May 2016 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 and who have an amenable mutation. Marketing applications have also been approved in several countries outside the EU, including Switzerland, Israel, and now Australia.

About Galafold™ and Amenable Mutations
Galafold® (migalastat) is a first-in-class chaperone therapy approved in Australia as a monotherapy for Fabry disease in patients with amenable mutations. Galafold 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) was used to classify more than 800 known *GLA* mutations as "amenable" or "not amenable" to treatment with Galafold. The Australian label includes 331 *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.

Amicus expects to submit additional updates to the Australian label as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

Australia 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 the indications, method of administration, special warnings, drug interactions and adverse drug reactions, please see the Australian Prescribing Information for Galafold available from the TGA website at https://www.tga.gov.au/product-information-pi.

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 biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating 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 clinical development of our product candidates, the prospects and timing of the potential regulatory and pricing approval of our product candidates. 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 and pricing 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: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that we may not be successful in commercializing Galafold in Europe and other geographies, including Australia; and the potential that we may not be successful in pricing and reimbursement discussions. 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. 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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CONTACTS:

Investors/Media:

Amicus Therapeutics

Sara Pellegrino, IRC

Senior Director, Investor Relations

spellegrino@amicusrx.com

(609) 662-5044

Primary Logo

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