Amicus Therapeutics Launches Galafold™ (Migalastat) for Treatment of Fabry Disease in Spain

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CRANBURY, N.J. and MADRID, Spain, Jan. 17, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, has commenced the commercial launch of the precision medicine Galafold in Spain following final pricing and reimbursement decisions. Galafold is now reimbursed in Spain 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.

"The commercial launch of Galafold in Spain adds to the tremendous momentum in expanding our launch throughout the EU, where we have now secured reimbursement in all five countries that have the largest Fabry populations within the EU," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "We believe that our pricing and reimbursement success throughout the EU, in addition to our global progress in securing additional approvals and completing new regulatory submissions, are a testament to the significant value of migalastat as the first oral precision medicine for people living with Fabry disease who have an amenable mutation."

Fabry disease is a genetic disease which causes deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the GLA gene. As a precision medicine, Galafold is designed to restore alpha-Gal A activity in patients who have amenable mutations (an estimated 35% to 50% of the Fabry population).

Dr. Roser Torra, Chief of Inherited Renal Diseases Unit. Nephrology Department, Fundación Puigvert, Barcelona stated, "There are unmet medical needs in Fabry disease, and oral Galafold represents a therapeutic alternative for Fabry patients with amenable mutations. Galafold is a new paradigm in precision medicine due to its innovative mechanism of action."

Jordi Cruz, Director of the Association of Mucopolysaccharidosis and Related Syndromes, MPS Spain, said, "It is great news each time a new treatment for rare diseases becomes available. The approval of this new treatment also represents a differentiated innovation in the treatment of this type of lysosomal storage disease. Patients with Fabry disease living in Spain who have an amenable mutation are fortunate to now have access to this oral precision medicine."

The European Commission granted the first approval for Galafold 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. Outside the EU, migalastat is approved in Switzerland, Israel, Australia, Canada and South Korea, with regulatory submissions under review in additional geographies.

About Galafold™ and Amenable Mutations
Galafold™ (migalastat) is a first-in-class chaperone therapy approved in the EU 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 1,000 known GLA mutations as "amenable" or "not amenable" to treatment with Galafold. The current EU label includes 313 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 Galafold. Amicus expects to submit additional updates to the label as additional GLA mutations are identified and tested in the Galafold Amenability Assay.

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 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 cornerstone of 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 future value driver of 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. 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 commercialization of Galafold, 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 we may not be successful in commercializing Galafold in Spain, Europe and other geographies or our other product candidates if and when approved. 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.

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Senior Director, Investor Relations
spellegrino@amicusrx.com
(609) 662-5044

Media:
Pure Communications
Jennifer Paganelli
jpaganelli@purecommunications.com
(347) 658-8290

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