Amicus Therapeutics Launches Galafold® (Migalastat) for Fabry Disease in Japan

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First Oral Precision Medicine for Fabry Patients with an Amenable Mutation Now Available in Japan

CRANBURY, N.J. and TOKYO, May 30, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) has initiated the commercial launch of the oral small molecule pharmacological chaperone Galafold® capsules 123mg (migalastat) for treatment of patients aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. Galafold is the first and only oral precision medicine for Fabry disease in Japan.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “The launch of Galafold in Japan, less than one year after our J-NDA submission, provides a significant opportunity for us to deliver this oral and differentiated precision medicine to amenable Fabry patients living in Japan. This is the result of Amicus employees, Japanese regulators, and the Fabry community working together to advance Galafold, in particular those physicians and patients who participated in the clinical studies of Galafold and their families. An estimated 850 people are currently known to be living with Fabry disease in Japan. We believe a significant portion of these Fabry patients have amenable mutations that could benefit from treatment with Galafold. Introducing Galafold in Japan is very important to our patient-focused vision to provide Galafold to Fabry patients with amenable mutations throughout the world as soon as possible. We also hope to have many future opportunities to deliver new medicines for people living with rare metabolic diseases in Japan.”

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 accumulated disease substrate in patients who have amenable mutations. An amenable mutation is one that is responsive to therapy with migalastat based on a proprietary *in vitro* assay (Galafold Amenability Assay).

Prof. Toya Ohashi, Jikei University, stated, “The launch of Galafold presents a differentiated treatment option that is good news for many Fabry patients in Japan. Significant unmet need remains for people living with Fabry disease, and I look forward to offering a new choice for patients with amenable mutations. Galafold has a unique mechanism of action that has demonstrated compelling results in naïve and treatment-experienced Fabry patients who have amenable mutations.”

Mr. Hisao Harada, Chair of the Japan Fabry Disease Patients and Family Association (JFA) commented, “I welcome the introduction of Galafold as an oral precision medicine and the first new Fabry treatment option in more than a decade for a number of Fabry patients in Japan who have amenable mutations."

An estimated 850 people in Japan are living with Fabry disease. Japan represented approximately 13% of the $1.3 billion global Fabry ERT sales generated in 2017.[1] Galafold was approved by the Ministry of Health, Labour and Welfare (MHLW) under the priority review scheme allowed for an Orphan Drug. The approval was based on clinical data from completed clinical studies of Galafold, including 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 a Phase 1 study that evaluated the pharmacokinetics of migalastat in Japanese volunteers.

Galafold is currently reimbursed in 19 countries on a commercial basis or through expanded access programs (EAPs). 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. Outside the EU, marketing applications have been approved in six markets, including Australia, Canada, Israel, South Korea, Switzerland, and now Japan. Approvals of Galafold are currently pending in the U.S. and Taiwan.

About Galafold and Amenable Mutations

Galafold® capsules 123 mg (migalastat) is a first-in-class chaperone therapy approved in Japan 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. Amicus estimates that 35%-50% of Fabry patients globally may have amenable genetic mutations, and amenability rates within this range vary by geography.

Healthcare providers in Japan 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 website as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

Important Japanese Safety Information

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not indicated for use in patients with a nonamenable mutation.

- The efficacy and the safety of concomitant use with enzyme replacement therapy has not been established. GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment. The safety and efficacy of GALAFOLD in low birth weight infants, neonates, nursing infants, infants and children have not yet been established.
- Migalastat exposure is affected by food, therefore it should not be taken within 2 hours before and after food.
- Patients should be observed carefully, and caution should be taken in the administration in the elderly population.

- 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, and consider the treatment only in case that the benefit from migalastat is judged to exceed the risk during pregnancy. Nursing mothers should be instructed not to breast-feed if they are taking migalastat or to discontinue migalastat if they do breast-feed
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- Patients should be monitored based on their course including renal and cardiac function and clinical laboratory test during migalastat treatment. In case no effect is observed in the migalastat treatment, changing treatment should be considered.
- OVERDOSE: Headache and dizziness were the most common adverse reactions reported at doses of migalastat of up to 1250 mg and 2000 mg, respectively, administrated in healthy subjects in the overseas clinical studies.
- 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 Japan package insert.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including dosage and administration, precautions, drug interactions and adverse drug reactions, please see the Japan package insert for Galafold available at http://www.pmda.go.jp/PmdaSearch/iyakuDetail/ResultDataSetPDF/112604_39990C4M1020_1_01

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 lead biologics program in the Amicus pipeline is AT-GAA, 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.

[1]Company filings and Amicus estimates

Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to approval and commercialization of Galafold in Japan. 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 pricing regulatory authorities, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, and the potential that we may not be successful in commercializing Galafold in Japan. 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, 2017. 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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