



Amicus Therapeutics Receives Marketing Authorization for Galafold® (migalastat) for Fabry Disease in Argentina

August 2, 2019

First Oral Precision Medicine Approved for People Living with Fabry Disease with an Amenable Mutation in Argentina

First Amicus Regulatory Approval in Latin America

CRANBURY, N.J., and BUENOS AIRES, Argentina, Aug. 02, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the Agency of Medicines, Food and Medical Devices (ANMAT) in Argentina has granted approval for the first-in-class, oral small molecule pharmacological chaperone Galafold® (migalastat) as a monotherapy for the treatment of people 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 approved in Argentina.

Amicus is working with a local partner, Pint Pharma, to complete the requirements to launch Galafold® in the coming months. Argentina represents the first approval for Galafold® in Latin America. Additional submissions for marketing authorizations are under review or planned in this important region.

"The approval of Galafold® in Argentina marks a significant step for our oral precision medicine for Fabry disease in Latin America," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics. "With more than 400 people known to be living with Fabry disease in Argentina, we look forward to collaborating with Pint Pharma, to bring our patient-dedicated approach to the country. As many thousands more are estimated to be living with Fabry in Latin America as a whole, Amicus and our partners are committed to supporting people affected by the disease throughout the region, and we believe a significant portion may have amenable mutations that could benefit from treatment with Galafold®."

David Munoz, Chief Executive Officer of Pint Pharma, stated, "The approval of Galafold® represents a major milestone for patients living with Fabry disease in Argentina and the region. We are honored to partner with Amicus and are committed to bringing a patient focused vision by supporting early and efficient access to innovative treatments."

The Argentinian label for Galafold® includes over 350 GLA mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay. It is estimated that between 35% and 50% of people diagnosed with Fabry disease may have an amenable GLA mutation.

Hernan Amartino, MD, Head of the Child Neurology Department at Hospital Universitario Austral, Buenos Aires, Argentina and Investigator in a migalastat clinical study, stated, "The approval and pending availability of Galafold® is great news for many Fabry patients in Argentina. Galafold® is the only approved oral therapy for Fabry disease with a mechanism of action designed to enable alpha-GAL A enzyme activity for patients with an amenable mutation. Galafold® has demonstrated meaningful results in previously untreated and treatment-experienced Fabry patients who have amenable mutations, and at one capsule every other day, at the same time of day, it is exciting for these patients to have a new choice of treatment."

Florencia Braga Menendez, Director of Patient Affairs of Fundación Investigar, an organization committed to access to healthcare in Argentina, added, "We welcome the approval of Galafold® as the first oral precision medicine and first new treatment option in more than a decade, for people living with Fabry disease who have an amenable mutation in Argentina. We are grateful to those who invest in researching and bringing hope to families that await treatment for so many rare diseases. Even the promise of a new treatment is powerful – it makes us feel less invisible. Today is a big day for Fabry patients and for the advancement of precision medicine in Argentina."

In addition to Argentina, Galafold® is approved in Australia, Canada, the European Union, Israel, Japan, South Korea, Switzerland and the U.S.

About Galafold® and Amenable Mutations

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. In patients with an amenable mutation, Galafold® (migalastat) works by stabilizing the body's own dysfunctional enzyme, so it can enter the lysosome. Once inside the lysosome, Galafold® detaches from the enzyme, allowing it to break down substrate. An amenable mutation is one that results in an enzyme that can be stabilized by Galafold®. Amenability is determined based on a proprietary *in vitro* assay (Galafold Amenability Assay).

Healthcare providers in Argentina may access the website www.galafoldamenabilitytable.com/ar to identify which mutations are categorized as "amenable" or "not amenable" to Galafold®. Amicus, through its partner Pint Pharma, expects to submit additional updates to the website as additional GLA mutations are identified and tested in the Galafold Amenability Assay.

Important Argentine 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 non-amenable 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, administered 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 Argentinian Galafold® 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 Argentina package insert for Galafold®.

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 on [Twitter](#) and [LinkedIn](#).

About Pint Pharma

Pint Pharma International SA is a company registered under Swiss laws, having its registered office at Route de Chenaux 9, 1091 Bourg-en-Levaux, Switzerland, and is devoted to the development, registration, and commercialization of specialty based treatments. Pint Pharma benefits from leaders with extensive experience in the pharmaceutical sector and who are based strategically throughout Latin America and Europe. Pint Pharma has a long track record of developing strong relationships with global pharmaceutical and healthcare companies. Pint Pharma strives to be the first Pan-Latin American provider of innovative and high value-added treatments within Rare Diseases, Specialty Care, and Oncology.

Forward Looking Statement

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 Argentina. 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, 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 the Argentina, the potential that Galafold® will not be reimbursed in Argentina, the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete all of our commercialization and manufacturing activities. 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 news release to reflect events or circumstances after the date hereof.

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