



May 2, 2017

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

CRANBURY, N.J. and PARIS, May 02, 2017 (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 France following the publication of the price in the Official Journal. Galafold is now reimbursed in France and dispensed via retail pharmacists as a therapy 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 France is a significant milestone for the Fabry community," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "Physicians and their Fabry patients in France 16 years and older who have an amenable mutation will have the opportunity to choose the first oral treatment as well as the first precision medicine for their Fabry disease. We believe that our successful pricing and reimbursement in France and other major EU markets is a testament to the significant value of our oral precision medicine, and reflects significant progress with the country-by-country processes to launch Galafold throughout the EU."

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).

Prof. Dominique P. Germain, MD, PhD, Division of Medical Genetics at the University of Versailles and Assistance Publique - Hôpitaux de Paris stated, "As a treating physician, and principal investigator for France in the clinical studies of Galafold for more than a decade, I am pleased that Fabry patients in France will now have access to this important new paradigm of precision medicine. Galafold has a unique mechanism of action that reflects a deep understanding of the underlying genetics of Fabry disease. More than 300 mutations that are amenable to this chaperone therapy have been identified. I believe that Galafold has significant potential to address unmet medical needs and represents a novel treatment for Fabry patients with amenable mutations. Galafold is also an excellent example of patient-focused drug development and precision medicine within the field of metabolic disease."

Mme Bedreddine, President of the French Fabry patient association (APMF), adds, "Each time a new treatment is found, it's very good news, especially where orphan diseases are concerned. People in France living with Fabry disease who have an amenable mutation consider themselves fortunate to now have access to the oral precision medicine. Furthermore, each new treatment represents a step forward in providing additional options for people living with Fabry."

The European Commission granted full approval for 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 and who have an amenable mutation.

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 800 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 m²). 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 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 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 preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. 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 authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, 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 it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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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Source: Amicus Therapeutics, Inc

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