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

First Oral Precision Medicine for Fabry Disease with Broad Label for Fabry Patients with Amenable Genetic Mutations

CRANBURY, N.J., Sept. 14, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) announced that Health Canada has approved the oral precision medicine Galafold for long-term treatment of adults with a confirmed diagnosis of Fabry disease [deficiency of alpha-galactosidase (alpha-Gal A)] and who have an alpha-Gal A mutation determined to be amenable by an *in vitro* assay. Following the Health Canada approval, Amicus expects to make Galafold available to Canadian patients in the coming weeks.

"The approval of Galafold represents a significant step forward for the Canadian Fabry community," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "We look forward to launching Galafold in Canada to further advance our mission of expanding global access to Galafold for people living with Fabry disease who have amenable mutations."

Health Canada approved Galafold 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 and overall body of evidence. Fabry 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).

"Following more than a decade of experience treating Fabry patients with Galafold in the clinical setting, I am pleased that Fabry patients in Canada who have amenable mutations will soon have access to this important and differentiated medicine," said Dr. Daniel Bichet, Full Professor and Section Head, Renal Function & Transport Physiology, University of Montreal, and Principal Investigator for Canada in the Galafold clinical studies. "Galafold has a unique mechanism of action that is rooted in the underlying genetics of Fabry disease. With approximately 270 mutations identified as amenable to this chaperone therapy in Canada, there are many patients who could potentially benefit from this new treatment option."

Julia Alton, Executive Director of the Canadian Fabry Association, stated, "Following Health Canada's approval of Galafold, we are delighted that the Fabry community in Canada will soon have access to the first new Fabry treatment option in nearly fifteen years. Amicus has partnered with the Fabry community for more than a decade to incorporate the needs of patients and physicians into the Galafold development process and we look forward to the differentiated treatment choice that Galafold will offer to Fabry patients in Canada who have amenable mutations."

About Galafold™ and Amenable Mutations
Galafold™ (migalastat) is a first-in-class chaperone therapy approved in Canada 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 Canadian label includes 270 *GLA* mutations that have been identified and determined to be amenable in Canada based on the Galafold Amenability Assay.

Healthcare providers in Canada 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 Canadian label as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

Important Canadian 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.

- Clinical data supporting the effectiveness of Galafold for the treatment of Fabry disease patients with amenable mutations are limited. In clinical trials, individual response to Galafold treatment varied considerably among patients with amenable mutations. Patients should be assessed for treatment response or failure when initiating Galafold, and monitored periodically thereafter (every 6 months or more frequently) throughout the treatment
- Galafold is not recommended for use in patients with a non-amenable mutation. Galafold may result in a net loss of α-Gal A activity in patients with non-amenable mutations, potentially worsening the disease condition.
- Galafold is not intended for concomitant use with enzyme replacement therapy.
- The patient should be advised to carefully adhere to the recommended dosing regimen of Galafold [one 123 mg migalastat capsules every other day (QOD)]. A higher dose or shorter dosing interval may result in a loss of efficacy, potentially worsening the disease condition
- Galafold should not be used in patients with severe renal insufficiency, defined as having an estimated glomerular filtration rate (eGFR) less than 30 mL/min/1.73m^2 , due to a significant increase in the exposure to migalastat and prolonged half-life of migalastat. This may result in a net loss of α-Gal A activity, potentially worsening the disease condition.
- The safety and efficacy of GALAFOLDTM in pediatric patients have not been established
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- Galafold should not be used by pregnant women and is not recommended in women of childbearing potential not using contraception.
- While taking Galafold, effective birth control should be used. Galafold should not be used in breast-feeding women. It is not known whether Galafold is excreted in human milk.
- Galafold is contraindicated for use in patients who are hypersensitive to this drug or to any ingredient in the formulation or component of the container
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache. For a complete list of adverse reactions, please review the Canadian Product Monograph.
- 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 Canadian Prescribing Information for Galafold available from the Health Canada website here.

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, 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 Canada; 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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