



Amicus Therapeutics Honors Fabry Disease Awareness Month and International Pompe Day

April 17, 2018

CRANBURY, N.J., April 17, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) is participating in several activities in April to honor Fabry Disease Awareness Month and International Pompe Day. The Fabry community has designated the month of April to honor and recognize those who living with the disease and to promote greater awareness. The goal of International Pompe Day on April 15 was to foster global awareness of Pompe disease.

In recognition of Fabry and Pompe awareness, Amicus is participating in the following:

- United Pompe Foundation's (UPF) 7th [Annual Late-Onset Pompe Disease Patient Meeting](#), hosted by the Duke Pompe Disease Clinical and Research Program, Durham, NC, April 6-7
- Annual [Fabulous Fabry Females Meeting](#), Emory University, Atlanta, GA, April 22
- New Fabry disease educational websites launched by Amicus:
 - [FabryFacts.com](#): a global educational resource providing information and resources about Fabry disease to support healthcare professionals in diagnosing and caring for people with Fabry disease. More information is available at [www.fabryfacts.com](#).
 - [FabryConnect](#): an interactive website for people living with Fabry disease to learn, share, and connect with the Fabry community. More information is available at [www.fabryfacts.com/fabryconnect](#).

"In the Amicus spirit of friendship, we join the Fabry and Pompe disease communities in supporting several activities to drive awareness during the month of April," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "Each day at Amicus, through our research and development efforts as well as our broader, employee-driven *Healing Beyond Disease* initiatives, we seek to bring innovation and hope to all people living with rare metabolic diseases."

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 Pompe Disease

[Pompe disease](#) is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

About Healing Beyond Disease

Healing Beyond Disease is a cross-functional initiative that was created and implemented by the employees of Amicus Therapeutics to enhance and expand the company's positive effect on people living with rare diseases. Launched in 2018, *Healing Beyond Disease* comprises elements of volunteerism, mentorship, philanthropic giving, innovation and accessibility - each inspired by and adaptive to rare disease communities. For more information please visit the [Amicus corporate website](#).

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 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. 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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