



April 9, 2015

## Amicus Therapeutics Honors Fabry Disease Awareness Month and International Pompe Day

CRANBURY, N.J., April 9, 2015 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, will support several activities surrounding Fabry Disease Awareness Month and International Pompe Day. The Fabry community has designated the month of April to honor and recognize those who are affected by the disease and to promote greater awareness of the disease. The goal of the [Second International Pompe Day](#) on April 15 is to foster international awareness of Pompe disease.

In collaboration with the Fabry and Pompe communities, Amicus will sponsor and participate in the following events:

- Fabry Support and Information Group's (FSIG) Female Fabry Get Togethers in Minneapolis, MN, on April 11; and Philadelphia, PA on April 18. For more information please contact FSIG at 660-463-1355 or visit [www.fabry.org](http://www.fabry.org)
- United Pompe Foundation's (UPF) Late-Onset Pompe Disease Patient Meeting hosted by the Duke Pompe Disease Clinical and Research Program, Durham, NC, from April 10-11. For more information please visit [www.dukechildrens.org/pompe](http://www.dukechildrens.org/pompe)
- FSIG's Fabry Fun Walk/Run in St. Louis, MO, on April 25. For more information please contact FSIG at 660-463-1355 or [info@fabry.com](mailto:info@fabry.com)
- All-employee Lunch and Learn at Amicus featuring guest speaker Jerry Walter, Founder of the National Fabry Disease Foundation

"We look forward to collaborating with our partners in the Fabry and Pompe communities to help advance disease education surrounding the significant unmet needs that exist for people living with both of these diseases," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "It is our vision at Amicus to improve the lives of individuals with rare and orphan diseases through our enduring commitment to patient centricity as well as our own drug development efforts."

### About Fabry Disease

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A, referred to here as alpha-Gal). The primary biological function of alpha-Gal 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 are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal activity lead to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart attack and stroke. It is currently estimated that Fabry disease affects approximately 5,000 to 10,000 people worldwide.

### About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called acid alpha-glucosidase (GAA). The role of GAA within the body is to break down lysosomal glycogen, the form of sugar stored in living cells for use as energy. Reduced or absent levels of GAA activity lead to the accumulation of glycogen in the affected tissues, including the heart, skeletal muscles (including those involved with breathing), liver, and nervous system. This accumulation of glycogen is believed to cause progressive muscle weakness and respiratory insufficiency in individuals with Pompe disease. Pompe disease affects an estimated 5,000 to 10,000 individuals worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression.

### About Amicus Therapeutics

[Amicus Therapeutics](#) (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage disorders. Amicus' lead programs in development include the small molecule pharmacological chaperone [migalastat](#) as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS-1.

## 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 Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus 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 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 the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical 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 may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical 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, 2014. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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