Amicus Therapeutics Provides U.S. Regulatory Update for Migalastat Monotherapy

CRANBURY, N.J., Oct. 2, 2015 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, today announced additional regulatory guidance from the U.S. Food and Drug Administration (FDA) on the oral small molecule pharmacological chaperone migalastat for the treatment of Fabry disease.

Amicus has received final FDA minutes from the September pre-NDA meeting and has conducted additional follow-up interactions with the Agency this week. In conjunction with the Agency, Amicus is further evaluating several U.S. pathways including potentially generating additional data on migalastat's effect on gastrointestinal symptoms in Fabry disease to support submission requesting full approval as well as a Subpart H strategy. In addition, the Agency has requested further integration of existing clinical data across studies which will require more time to complete. Based on this guidance from the FDA, Amicus does not anticipate being in a position to submit the NDA for migalastat monotherapy in the United States by the end of this year. The timing of an NDA submission will be based on the determination of the optimal regulatory pathway.

"Amicus remains committed to making migalastat available to Fabry patients with amenable mutations in the U.S. as rapidly as possible. We are appreciative of the FDA's ongoing collaboration in this program," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics.

About Fabry Disease

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). 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. 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.

About Amicus Therapeutics

Amicus Therapeutics, Inc. (Nasdaq:FOLD) is a 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 next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS I.

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 outcomes 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 and Form 10-Q for the quarter ended June 30, 2015. 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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