U.S. FDA Grants Breakthrough Therapy Designation (“BTD”) to Amicus’ AT-GAA in Late Onset Pompe Disease

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First BTD Granted for a Second Generation Lysosomal Storage Disorder Therapy

First BTD for an Investigational Treatment in Pompe Disease

CRANBURY, N.J., Feb. 25, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has granted to Amicus a Breakthrough Therapy Designation (“BTD”) to AT-GAA for the treatment of late onset Pompe disease, an inherited lysosomal storage disorder caused by the deficiency of an enzyme known as acid alpha-glucosidase (GAA). AT-GAA is the first ever investigational product for Pompe disease to receive BTD. AT-GAA is a novel treatment paradigm consisting of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, co-administered with AT2221, a pharmacological chaperone.

Breakthrough Therapy Designation was instituted by the FDA in 2012 to expedite the development and review of drugs that target serious conditions. To receive BTD there must be preliminary clinical evidence indicating that the drug may demonstrate substantial improvement on a clinically significant endpoint over available therapies. The designation includes all of the Fast Track program features, as well as more intensive FDA guidance and discussion. The BTD will facilitate multidisciplinary, comprehensive discussions of the AT-GAA development program with the FDA, including planned clinical trials and plans for expediting manufacturing development strategy.

The BTD for AT-GAA is based on clinical efficacy results from the ongoing ATB200-02 Phase 1/2 clinical study, including improvements in six-minute walk distance in late onset Pompe patients and comparison to natural history of treated patients.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, “There is an urgent need for new, second generation therapies for people living with lysosomal storage disorders, especially in a disease like Pompe. This important Breakthrough Therapy Designation from the FDA reflects the clinical data for our novel Pompe treatment paradigm AT-GAA. The BTD here also is based on the significant unmet need that remains for people living with Pompe disease, despite an approved therapy. We have been very pleased with the level of collaboration among the Amicus team, physicians, patients and the FDA in advancing AT-GAA. This BTD, together with our results from the Phase 2 study and ongoing PROPEL pivotal study, support our strategy to advance AT-GAA as quickly as possible with the potential to become the new standard of care for all persons living with Pompe disease.”

Amicus continues to expect that the pivotal PROPEL study (also referred to as ATB200-03) will be needed to support full approval of AT-GAA. Based on regulatory feedback from both the U.S. FDA and the European Medicines Agency (EMA), the Company expects the AT-GAA development plan, including a planned pediatric study, to support a broad indication, for ERT-switch and treatment-naïve Pompe patients.

For more information about the PROPEL study visit www.pompestudy.com.

For more information about Breakthrough Therapy Designation visit www.fda.gov.

About AT-GAA
AT-GAA is an investigational therapy that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In preclinical studies, AT-GAA was associated with increased tissue enzyme levels, reduced glycogen levels in muscle, and improvements in muscle strength. A global Phase 1/2 study (ATB200-02) is ongoing to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of AT-GAA.

Amicus has also initiated PROPEL, a global Phase 3 clinical study (also known as ATB200-03) of AT-GAA in adult patients with late onset Pompe disease. PROPEL is a 52-week, double-blind randomized study designed to assess the efficacy, safety and tolerability of AT-GAA compared to the current standard of care, alglucosidase alfa, an enzyme replacement therapy (ERT). More information, including a list of participating sites, is available at www.clinicaltrials.gov: NCT03729362.

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. The 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 Amicus Therapeutics
Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company’s website at www.amicusrx.com, and follow us on Twitter and LinkedIn.

Forward-Looking Statements
This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including

statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate AT-GAA for the treatment of Pompe and the potential implications on these data for the future advancement and development of AT-GAA. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” "confidence," "encouraged," “potential,” “plan,” “targets,” “likely,” “may,” “will,” “would,” “should” and “could,” and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of AT-GAA, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize AT-GAA. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 and Quarterly Report on 10-Q for the Quarter ended September 30, 2018. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

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