Amicus Therapeutics Announces First Patient Dosed in Phase 3 PROPEL Pivotal Study of AT-GAA in Patients with Pompe Disease

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Global Study to Assess AT-GAA Compared to Standard-of-Care Enzyme Replacement Therapy (ERT) over 52 Weeks

CRANBURY, N.J., Dec. 20, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced the dosing of the first patient in a global phase 3 clinical study (ATB200-03, or PROPEL) 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).

All participants randomized to AT-GAA in the PROPEL study will receive drug manufactured at the 1000L scale intended for clinical and commercial supply. Amicus also expects to initiate a smaller, open-label study of AT-GAA in pediatric patients in 2019.

"The initiation of our global PROPEL study is a true example of our capabilities to discover, develop and manufacture promising therapies, and deliver them to patients as quickly as we can," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "With the first patient treated in this important study, we are now able to provide access to AT-GAA to many more adults with late onset Pompe disease. Importantly, this study is the first to offer AT-GAA manufactured at our commercial scale following many years of diligent work on the CMC and manufacturing side to produce biocomparable material that we can now supply for the study and future global Pompe population. People living with Pompe disease are urgently seeking new options, and we hope that the treatment experience in this study will enable global regulatory submissions."

Pompe disease is an inherited lysosomal storage disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to the accumulation of glycogen in cells and causes progressive muscle weakness throughout the body, affecting several vital tissues of the body. 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.

Tahseen Mozaffar, MD, Professor and Chair of Neurology and Director, Neuromuscular Program at UC Irvine and Principal Investigator in the PROPEL study stated, "The PROPEL study provides us with the opportunity to build upon the very compelling data set from the earlier Phase 1/2 clinical study. The PROPEL study is well-designed, with an active comparator arm and several important assessments of efficacy, including the gold standard six-minute walk test. I look forward to treating more patients with AT-GAA in this study."

The PROPEL study is expected to enroll approximately 100 participants (ERT-experienced and ERT-naïve) at up to 90 global sites. Participants will be randomized 2:1 to receive investigational AT-GAA (ATB200 co-administered with the oral chaperone AT2221) or standard of care ERT co-administered with an oral placebo for a 52-week double-blind primary treatment period. Following this primary treatment period, all participants will be eligible to receive AT-GAA in a long-term, open-label extension study.

The PROPEL study is designed to assess superiority of AT-GAA compared to alglucosidase alfa. The primary efficacy endpoint is change in six-minute walk distance from baseline to Week 52. Secondary endpoints include respiratory measures and additional measures of muscle function and muscle strength. More information, including a list of participating sites, is available at www.clinicaltrials.gov: NCT03729362.

Based on regulatory feedback from both the U.S. FDA and European Medicines Agency (EMA), the PROPEL study is expected to support approval for a broad indication, including ERT switch patients and treatment naive, as well as the pediatric population. Enrollment in the PROPEL study is expected to complete in 2019.

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.

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 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 a global Phase 3 study to investigate AT-GAA for the treatment of Pompe and the potential implications of this study 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 previously reported 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 manufacture or 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.

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations & Corporate Communications
spellegrino@amicusrx.com
(609) 662-5044

Media:
Pure Communications
Jennifer Paganelli
jpaganelli@purecommunications.com
(347) 658-8290

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