Amicus Therapeutics Provides Updates on Clinical, Regulatory and Manufacturing Advancements for AT-GAA for Pompe Disease

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123 Patients Enrolled in Phase 3 PROPEL Study – Enrollment Completed in 4Q2019

Company Plans to Apply for and Initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020 with Addition of Full Clinical Results in 1H2021 to Support Full Approval under Fast Track Designation

Promising Innovative Medicine (PIM) Designation Issued by British MHRA with Potential for Early Access for Pompe Patients in United Kingdom Based on Phase 2 Results

Biologics Manufacturing with WuXi Biologics on Track with PPQ Runs at Commercial Scale

CRANBURY, N.J., Jan. 07, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced clinical, regulatory and manufacturing advancements for AT-GAA, its investigational therapy for Pompe disease. Pompe disease is an inherited lysosomal disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells and is characterized by severe muscle weakness that worsens over time.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, “Our team’s efforts to bring AT-GAA to all patients living with Pompe around the world continues at a momentous pace and with great passion. We know that there is an urgent need for new, second-generation therapies for people living with lysosomal disorders, especially in Pompe disease. Regulatory agencies in the U.S. and now the U.K., have recognized the highly differentiated clinical data set and the great potential for AT-GAA to meet the currently unmet medical needs in Pompe disease through the granting of Breakthrough Therapy Designation and the Promising Innovative Medicine Designation, respectively. Amicus now plans to apply for and initiate a rolling submission of the AT-GAA BLA in the U.S. later this year. These designations, together with the results from the Phase 2 study and the now fully enrolled 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.”

Achieved Full Enrollment of Phase 3 PROPEL Study
During the fourth quarter of 2019, Amicus completed and exceeded patient enrollment in the global Phase 3 PROPEL clinical study for Pompe disease. A total of 59 clinical sites have enrolled 123 patients globally.

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) in adults with late-onset Pompe disease (LOPD). The PROPEL study is intended to assess superiority of AT-GAA compared to alglucosidase alfa in both ERT switch and ERT naïve patients. 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.

Rolling Biologics License Application (BLA) for AT-GAA
Amicus plans to apply for and initiate a rolling submission of the BLA for AT-GAA in late-onset Pompe disease and to complete final submission in the first half of 2021. A rolling submission allows the Company to submit portions of the regulatory application to the U.S. Food and Drug Administration (FDA) as they are completed, rather than waiting until every section of the BLA is complete to submit the entire application for review.

The U.S. FDA previously granted Breakthrough Therapy Designation to AT-GAA for the treatment of late onset Pompe disease based on clinical efficacy results from the Phase 1/2 clinical study, including improvements in six-minute walk distance, an integrated measure of disease progression that evaluates the cardiopulmonary and musculoskeletal systems, as well as comparison to natural history of treated patients.

Promising Innovative Medicine (PIM) designation for AT-GAA
The British Medicines and Healthcare products Regulatory Agency (MHRA) has issued a Promising Innovative Medicine (PIM) designation for AT-GAA in late onset Pompe disease. A PIM designation signifies that a clinical program may be eligible for the Early Access to Medicines Scheme (EAMS), which works to provide patients with life threatening or seriously debilitating conditions access to medicines with the potential to address an unmet medical need.

PIM designation for AT-GAA is based on clinical efficacy results from the Phase 1/2 clinical study, including improvements in six-minute walk distance, an integrated measure of disease progression that evaluates the cardiopulmonary and musculoskeletal systems, as well as comparison to natural history of treated patients. Amicus is evaluating the EAMS program and will provide a future update.

Biologics Manufacturing with WuXi Biologics on Track
Process performance qualification (PPQ) runs have been initiated with the Company’s key strategic partner, WuXi Biologics. The PPQ runs will serve as the foundation for the Chemistry, Manufacturing, and Control (CMC) module for a BLA submission. The manufacturing focus remains on AT-GAA inventory build, which will support: the clinical studies underway, further studies including in pediatrics, as well as to supply the early commercial inventory. WuXi Biologics will enable and support Amicus with its dual sourcing strategy and manufacture at two sites across their network, including its new facility in Ireland, which remains on-track.

About AT-GAA

AT-GAA is an investigational therapy that consists of cipaglucosidase alfa (ATB200), a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake into cells, co-administered with miglustat (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 is also conducting an ongoing 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). The primary endpoint is six-minute walk distance, an integrated measure of disease progression that evaluates the cardiopulmonary and musculoskeletal systems essential to performing the activities of daily living for patients with Pompe disease. More information, including a list of participating sites, is available at www.clinicaltrials.gov: NCT03729362. In addition, Amicus is enrolling an open-label, uncontrolled, multicenter study to evaluate the PK, safety, efficacy, and PD of AT-GAA in pediatric patients aged 12 to <18 years with LOPD More information, including a list of participating sites, is available at www.clinicaltrials.gov: NCT03911505

About Pompe Disease
Pompe disease is an inherited lysosomal disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA levels lead 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.

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