Amicus Therapeutics Initiates Rolling Biologic License Application to the U.S. Food and Drug Administration for AT-GAA in Late-Onset Pompe Disease

December 1, 2020

On Track for Completing the BLA Submission in 1H2021

All PPQ Drug Substance and Drug Product Activities for Manufacturing Now Successfully Completed

Data from Phase 3 Propel Study Expected 1Q2021

CRANBURY, N.J., Dec. 01, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced the initiation of the rolling Biologics License Application (BLA) submission to the U.S. Food and Drug Administration (FDA) for AT-GAA, its investigational two-component therapy for the treatment of late onset Pompe disease (LOPD). The rolling submission allows the Company to submit portions of the regulatory application to the FDA as they are completed, rather than waiting until every section of the BLA is complete to submit the entire application for review. The FDA earlier this year authorized Amicus to proceed with a Rolling BLA submission.

AT-GAA is an investigational therapy that consists of cipaglucosidase alfa (ATB200), a unique enzyme replacement therapy with optimized carbohydrate structures, administered in conjunction with miglustat (AT2221), an orally administered stabilizer of cipaglucosidase alfa. Amicus has submitted the nonclinical component of the cipaglucosidase alfa BLA and is on-track to submit the chemistry, manufacturing and controls (CMC) component, and the final clinical module in the first half of 2021. Cipaglucosidase alfa is to be used in conjunction with miglustat, which will be a separate NDA submitted at the same time as the remainder of the cipaglucosidase alfa BLA.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, “This submission represents a major milestone for Amicus and the entire Pompe community. The rolling BLA for this investigational therapy supports our belief in the significant unmet need in Pompe disease and further supports our strategy to advance AT-GAA as quickly as possible. Today’s announcement, along with prior designations granted by regulatory agencies and the growing body of clinical data continue to support our belief that AT-GAA has the potential to become the new standard of care for individuals living with Pompe disease. This submission puts us all one step closer to this goal.”

The U.S. FDA previously granted Breakthrough Therapy Designation to AT-GAA for the treatment of LOPD based on clinical efficacy results from the Phase 1/2 clinical study, including improvements in six-minute walk distance, as well as comparison to natural history of treated patients. Top-line data for the global Phase 3 PROPEL clinical study of AT-GAA in LOPD is on-track for the first quarter of 2021.

About AT-GAA

AT-GAA is an investigational two-component therapy that consists of cipaglucosidase alfa (ATB200), a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly bis-phosphorylated mannose-6 phosphate (bis-M6P) glycans, to enhance uptake into cells, administered in conjunction with miglustat (AT2221), a stabilizer of cipaglucosidase alfa. In preclinical studies, AT-GAA was associated with increased levels of the mature lysosomal form of GAA and reduced glycogen levels in muscle, alleviation of the autophagic defect and improvements in muscle strength. A global Phase 1/2 study (ATB200-02) is ongoing to evaluate the safety, tolerability, pharmacokinetics 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 (LOPD). 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 (ATB200-04). 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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