Amicus Therapeutics Announces Presentation and Posters at the 2021 MDA Clinical & Scientific Conference

March 15, 2021

PHILADELPHIA, March 15, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), today announced one oral presentation and two posters highlighting its development program for Pompe disease will be included at the 2021 MDA Virtual Clinical & Scientific Conference to be held March 15-18, 2021.

Oral Platform Presentation: Thursday, March 18, 5:30 – 5:45 p.m. ET

- *Efficacy and safety of AT-GAA (cipaglucosidase alfa/miglustat) versus alglucosidase alfa/placebo in late-onset Pompe disease (LOPD): A phase 3 trial (PROPEL)*
    - Presenter: Tahseen Mozaffar, MD, FAAN, University of California, Irvine, CA, USA

Poster Presentations:

- *Characterization of Response to Enzyme Replacement Therapy in Patients With Late-Onset Pompe Disease: A Retrospective Chart Review*
    - Presenter: Priya Kishnani, MD, MBBS, Duke University Medical Center, Durham, NC, USA

- *Enhancing Delivery of Acid Alpha-Glucosidase (GAA) to Skeletal Muscle in Pompe Disease (PD): Key Challenges and Attributes of AT-GAA*
    - Presenter: Nithya Selvan, Ph.D., Amicus Therapeutics, Philadelphia, PA, USA

The posters and presentation will be made available on the Amicus website following their respective presentations at the conference.

For more information on the 2021 MDA Virtual Clinical & Scientific Conference, please visit www.mdaconference.org.

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.

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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