U.S. FDA Accepts Filings for Amicus' AT-GAA for the Treatment of Pompe Disease

September 29, 2021

FDA Sets PDUFA Target Action Date of May 29, 2022 for the New Drug Application and July 29, 2022 for the Biologics License Application

On Track for MAA Submission in the Fourth Quarter of this Year

PHILADELPHIA, Sept. 29, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat for AT-GAA, the Company's investigational two-component therapy for the treatment of Pompe disease. Pompe disease is a rare genetic disease that causes premature death and has a debilitating effect on people's lives. The U.S. represents the single largest geography for Amicus to positively impact the lives of people with Pompe disease.

The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA. The BLA and NDA are based on the evaluation of the effects of AT-GAA in Pompe disease patients and its safety profile, which include data from the Phase 1/2 and Phase 3 PROPEL studies as well as data from the open-label extension study.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics Inc., stated, "The FDA's acceptance of these filings is an immensely important step forward for people living with Pompe disease and their families in the United States. Patients need new medicines as soon as possible. We will work with great urgency with the FDA as they review the applications over the course of the coming months. In parallel, we are diligently working towards additional regulatory submissions outside of the U.S. With today's announcement, we remain confident in the potential of this medicine to become the next standard of care in Pompe disease."

Previously, the U.S. FDA 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. The Marketing Authorization Application for AT-GAA is expected to be submitted in the EU in the fourth quarter of 2021. In June 2021, the U.K.'s Medicines and Healthcare Products Regulatory Agency (MHRA) granted AT-GAA a positive scientific opinion through the Early Access to Medicines Scheme (EAMS).

About AT-GAA

AT-GAA is an investigational two-component therapy that consists of cipaglucosidase alfa (ATB200), a 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.

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.

Forward-Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to top-line data from a global Phase 3 study to investigate AT-GAA for the treatment of Pompe Disease, the potential implications on these data for the future advancement and development of AT-GAA, and anticipated regulatory submissions. There can be no assurance that the FDA will grant approval for 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 Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully manufacture and 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, 2020 and Quarterly Report 10-Q for the quarter ended June 30, 2021. 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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