Amicus Therapeutics Receives Notification of PDUFA Date Extensions for AT-GAA

May 10, 2022

PHILADELPHIA, May 10, 2022 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has extended the review period by 90 days for the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA. The revised PDUFA action dates for miglustat and cipaglucosidase alfa are August 29, 2022 and October 29, 2022, respectively. The Company continues to expect the FDA to approve the applications together.

The FDA extended the PDUFA dates to allow additional time to review information submitted by the Company as part of its ongoing reviews. The extension of the review timeline was not related to requests for any additional clinical data. The Company also expects that the additional time will allow for the completion of the pre-license approval inspections necessary at the WuXi Biologics manufacturing site in China.

"We continue to work collaboratively with the FDA as it completes its review of the AT-GAA applications," said John F. Crowley, Chairman and Chief Executive Officer at Amicus Therapeutics. "We want to thank the FDA for its continued diligence during the review process. We remain deeply committed to bringing AT-GAA to as many people living with Pompe disease as quickly as possible and delivering on our promise to become the potential new standard of care."

Previously, the 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. In the European Union, the Marketing Authorization Applications were validated in the fourth quarter of 2021 and the Committee for Medicinal Products for Human Use (CHMP) opinion is expected in late 2022.

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 0 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 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 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, expectations regarding the FDA regulatory process, and the outcome of the FDA's review. 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, 2021 and Quarterly Report 10-Q for the quarter ended March 31, 2022. 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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