



U.S. FDA Defers Action on Filing for AT-GAA in Late-onset Pompe Disease

October 28, 2022

FDA Issues Deferred Action Letter on AT-GAA Regulatory Filing Due to the Inability to Conduct Required Manufacturing Site Inspection Prior to the PDUFA Action Date

Company is Now Actively Engaged with the Agency to Develop Plans and Logistics for a Pre-Approval Inspection

PHILADELPHIA, Oct. 28, 2022 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has deferred action on the Biologics License Application (BLA) for cipaglucosidase alfa, the biologic component of AT-GAA. Due to restrictions on travel related to COVID-19, the FDA was unable to conduct the required inspection of the WuXi Biologics manufacturing site in China during the review cycle. As a result, the FDA is deferring action on the application until the manufacturing site inspection is complete. The Company continues to expect the FDA to approve the two components of AT-GAA, including the BLA and New Drug Application (NDA) for miglustat, together.

The sole reason cited in the FDA-issued letter for the deferred action was the Agency's inability to complete the manufacturing facility inspection. While both applications remain under review, the FDA has not provided anticipated action date(s) as they continue to monitor the public health situation and travel restrictions in China. However, the Company is now actively engaged with the FDA on developing plans and logistics for a pre-approval inspection plan.

Under FDA guidance relating to pre-approval inspections during the COVID-19 pandemic, the Agency may defer action on a pending application when a facility inspection is necessary but cannot be completed by the PDUFA goal date due to travel restrictions, provided that no deficiencies have been identified and the application otherwise satisfies the requirements for approval.¹

"We are now one step away from the necessary approvals for AT-GAA in the U.S. We continue to believe this is a question of 'when' not 'if' AT-GAA will be approved and we will continue to work with great urgency to support the FDA's completion of the final plant inspection necessary for approval so that this important new treatment option is made available for people living with Pompe disease in the United States," said Bradley Campbell, President and Chief Executive Officer at Amicus Therapeutics, Inc. "We are also very pleased with the progress of the regulatory review in the EU and look forward to a Committee for Medicinal Products for Human Use ("CHMP") opinion by the end of the year. We remain committed to bringing AT-GAA to as many people living with Pompe disease around the world as quickly as possible."

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, where a pre-approval inspection is not required, the regulatory review is on track and the Committee for Medicinal Products for Human Use (CHMP) opinion is expected before year end.

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.

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 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 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 expectations regarding the FDA regulatory process, and the outcome of the FDA's review and FDA's ability to conduct a manufacturing site inspection. There can be no assurance that the FDA will grant approval for AT-GAA or the timing of any such approval. 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 June 30, 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.

References

1. U.S. Food and Drug Administration. Manufacturing, Supply Chain, and Drug Inspections - COVID-19. Available at: <https://www.fda.gov/drugs/coronavirus-covid-19-drugs/manufacturing-supply-chain-and-drug-inspections-covid-19> Last accessed: October 2022.

CONTACTS:

Investors:

Amicus Therapeutics
Andrew Faughnan
Executive Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:

Amicus Therapeutics
Diana Moore
Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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