Amicus Therapeutics Receives Positive CHMP Opinion for Pombiliti™ (cipaglucosidase alfa) for Late-Onset Pompe Disease

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CHMP Adopts Positive Opinion Based Upon Complete Review of all Pre-Clinical, Clinical Studies and CMC Data

CHMP Recommends Label for Long-Term Enzyme Replacement Therapy in Combination with Miglustat for both ERT-Experienced and Treatment-Naïve Adults Living with Late-Onset Pompe Disease

CHMP Opinion for Miglustat, the Oral Enzyme Stabilizer Component of AT-GAA, Expected 2Q 2023

PHILADELPHIA, Dec. 16, 2022 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending marketing authorization of cipaglucosidase alfa, a long-term enzyme replacement therapy (ERT) used in combination with miglustat for adults with late-onset Pompe disease (LOPD). A decision from the European Commission (EC) on cipaglucosidase alfa, the enzyme replacement therapy component of AT-GAA, is expected in the first quarter of 2023. Cipaglucosidase alfa will be commercialized under the brand name POMBILITI™. The Company expects a CHMP opinion of miglustat, the enzyme stabilizer component of AT-GAA, in the second quarter of 2023.

Late-onset Pompe disease is a rare, debilitating, and life-threatening lysosomal disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscles and other tissues. Disease severity ranges on a spectrum, but predominant manifestations are skeletal muscle weakness and progressive respiratory involvement.

"Today's positive CHMP opinion for Pombiliti™ (cipaglucosidase alfa) is a significant milestone and major step towards bringing this much needed new treatment for all adults living in the EU with late-onset Pompe disease. It is the realization of the work of so many individuals and teams dedicated to the mission of improving the lives of people living with Pompe disease," said John F. Crowley, Executive Chairman and Founder of Amicus Therapeutics, Inc.

"Our team has worked tirelessly over the past decade to develop this innovative therapy, which we believe has the potential to address many of the unmet medical needs in this disease. We are grateful for the dedication and support from the Pompe community who have helped advance this therapy, especially the patients, families, and physicians who participated in our clinical studies. Based on the strength of the label and our launch readiness, once both components are approved, we believe there is significant commercial opportunity for AT-GAA in Europe and around the world," said Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc.

"This significant milestone moves AT-GAA closer to the LOPD community, where there is a high medical need for novel treatment options across patients, including those naïve and experienced to current treatments," said Prof. Benedikt Schoser, Professor of Neurology at Ludwig-Maximillians-University of Munich LMU Department of Neurology. "The CHMP positive opinion and recommended indication reflect the robust data from AT-GAA's clinical development program and gives me further hope for the potential of this innovative treatment alternative for people living with LOPD."

AT-GAA is designed as a two-component therapy consisting of cipaglucosidase alfa, a long-term enzyme replacement therapy, administered in combination with miglustat, an oral enzyme stabilizer, for the treatment of adults with late-onset Pompe disease. Cipaglucosidase alfa is a recombinant human acid alpha-glucosidase enzyme (rhGAA) enriched with bis-mannose-6-phosphate designed to facilitate high-affinity uptake with retained capacity for processing into the most active form of the enzyme to break down glycogen.

The CHMP based its positive opinion on clinical data from the Phase 3 pivotal study (PROPEL), the only randomized, controlled trial in LOPD to include patients in the high unmet need ERT-experienced population, in addition to ERT-naïve patients. As anticipated and consistent with a recent opinion of another ERT in this disease space, the CHMP also determined cipaglucosidase alfa does not qualify as a New Active Substance (NAS).

About AT-GAA

AT-GAA is a two-component therapy that consists of cipaglucosidase alfa, a bis-M6P-enriched rhGAA which facilitates high-affinity uptake through the M6P receptor while retaining its capacity for processing into the most active form of the enzyme, and the oral enzyme stabilizer, miglustat, that's designed to minimize loss of enzyme activity in the blood. In clinical studies, AT-GAA was associated with demonstrated improvements in both musculoskeletal and respiratory measures.

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 lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. 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 and progressive respiratory involvement. Late-onset Pompe disease can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the skeletal muscles and muscles controlling breathing, that worsens over time.

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 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 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 regulatory process in the US and Europe, and the outcome of those regulatory reviews. There can be no assurance that the EMA will grant full approval for both components of AT-GAA or when any such approvals may occur. 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 full regulatory approval for, or successfully manufacture and commercialize AT-GAA once fully approved. 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 September 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.

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