Amicus Therapeutics Announces European Commission Approval for Pombiliti™ in Patients with Late-Onset Pompe Disease

March 27, 2023

Approved as a Long-Term Enzyme Replacement Therapy in Combination with Miglustat for All Adults Living with Late-Onset Pompe Disease

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

PHILADELPHIA, March 27, 2023 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced that the European Commission (EC) has granted approval for Pombiliti™ (cipaglucosidase alfa), a long-term enzyme replacement therapy (ERT) used in combination with miglustat for adults with late-onset Pompe disease (LOPD). The Company has submitted the previously requested analytical testing for miglustat, the enzyme stabilizer component of AT-GAA. The Committee for Medicinal Products for Human Use (CHMP) opinion for miglustat is expected in the second quarter of 2023.

“Late-onset Pompe disease is a rare, neuromuscular disorder that can have devastating consequences for patients and their families. The European Commission approval for Pombiliti is another 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 of Amicus Therapeutics, Inc.

"We are extremely pleased with the EC approval of Pombiliti, an innovative enzyme replacement therapy that is intended for use in combination with the oral enzyme stabilizer miglustat. We are grateful to the Pompe community who have helped advance this therapy, especially the patients, families, and physicians around the world who participated in our clinical studies. Given the strength of the label and our launch readiness, we believe there is significant commercial opportunity, and that AT-GAA has the potential to become the next standard of care in Pompe disease by redefining the therapeutic expectations of people living with Pompe disease and of their caregivers. Once both components are approved, we look forward to bringing AT-GAA to people in Europe living with LOPD as rapidly as possible,” said Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc.

The EC based its approval 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. The EC approval of Pombiliti follows the positive opinion previously granted by the Committee for Medicinal Products for Human Use (CHMP).

About AT-GAA

AT-GAA is an investigational 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.

Important Safety Information

Pombiliti (cipaglucosidase alfa) Important Safety Information

Posology and Method of Administration: Pombiliti must be used in combination with miglustat 65 mg hard capsules. The recommended dose of Pombiliti is 20 mg/kg of body weight every other week. The Pombiliti infusion should start 1 hour after taking miglustat capsules. ***Paediatric population:*** The safety and efficacy of Pombiliti in combination with miglustat therapy in paediatric patients less than 18 years old have not yet been established. No data are available. ***Contraindications*****:** Life-threatening hypersensitivity to the active substance, or to any of the excipients. Contraindication to miglustat. ***Anaphylaxis and infusion-associated reactions (IARs):*** Serious anaphylaxis and IARs have occurred in some patients during infusion and following infusion with Pombiliti. Premedication with oral antihistamine, antipyretics, and/or corticosteroids may be administered to assist with signs and symptoms related to IARs experienced with prior enzyme replacement therapy (ERT) treatment. Reduction of the infusion rate, temporary interruption of the infusion, symptomatic treatment with oral antihistamine, or antipyretics, and appropriate resuscitation measures should be considered to manage serious IARs. If anaphylaxis or severe allergic reactions occur, infusion should be immediately paused, and appropriate medical treatment should be initiated. The current medical standards for emergency treatment of anaphylactic reactions are to be observed and cardiopulmonary resuscitation equipment should be readily available. The risks and benefits of re-administering Pombiliti following

anaphylaxis or severe allergic reaction should be carefully considered, and appropriate resuscitation measures made available. ***Risk of acute cardiorespiratory failure in susceptible patients:*** Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk of serious exacerbation of their cardiac or respiratory compromise during infusions. Appropriate medical support and monitoring measures should be readily available during Pombiliti infusion. ***Immune complex-related reactions:*** Immune complex-related reactions have been reported with other ERTs in patients who had high IgG antibody titres, including severe cutaneous reactions and nephrotic syndrome. If immune complex-related reactions occur, discontinuation of the administration of Pombiliti should be considered and appropriate medical treatment should be initiated. The risks and benefits of re-administering Pombiliti following an immune complex-related reaction should be reconsidered for each individual patient. ***Contraception in females:*** Reliable contraceptive measures must be used by women of childbearing potential during treatment with Pombiliti in combination with miglustat, and for 4 weeks after discontinuing treatment. ***Pregnancy:*** Pombiliti in combination with miglustat therapy is not recommended during pregnancy. ***Breast feeding:*** It is not known if Pombiliti and miglustat are secreted in human breast milk. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from Pombiliti in combination with miglustat therapy taking into account the benefit of breast-feeding for the child and the benefit of therapy for the woman. ***Summary of the safety profile:*** The most commonly reported adverse reactions only attributable to Pombiliti were chills (4.0%), dizziness (2.6%), flushing (2.0%), somnolence (2.0%), chest discomfort (1.3%), cough, (1.3%), infusion site swelling (1.3%), and pain (1.3%). Reported serious adverse reactions only attributable to Pombiliti were urticaria (2.0%), anaphylaxis (1.3%), pyrexia (0.7%), presyncope (0.7%), dyspnoea (0.7%), pharyngeal oedema (0.7%), wheezing (0.7%), and hypotension (0.7%). Refer to SmPC for full list.

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 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 expectations regarding the regulatory process in Europe. 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, 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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