Amicus Therapeutics Receives the 2024 New Treatment Award for Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) at the 20th Annual WORLDSymposium™

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- Award Recognizes Approval of Amicus Therapeutics' Two-component Therapy for the Treatment of Late-onset Pompe Disease -

PRINCETON, N.J., Feb. 08, 2024 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), today announced that WORLD*Symposium*™ has awarded Pombiliti™ (*cipaglucosidase alfa-atga*) + Opfolda™ (*miglustat*) the 2024 New Treatment Award, which recognizes important achievements in advancing treatments for lysosomal diseases which have attained regulatory approval.

"We are honored that WORLD *Symposium* has recognized Pombiliti and Opfolda with the 2024 New Treatment Award," said Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc. "It is a testament to more than a decade of dedication and commitment from Team Amicus along with dozens of families, physicians, researchers, and advocates around the world who worked tirelessly to see this medicine approved. Their partnership has been pivotal in continuing our promise to lead the fight in rare diseases, and we share this award with the entire global Pompe disease community."

Pombiliti + Opfolda is the first and only two-component therapy approved by the U.S. Food and Drug Administration (FDA) for adults with late-onset Pompe disease weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT). Pombiliti + Opfolda is a unique two-component therapy. Pombiliti is a recombinant human GAA enzyme (rhGAA) naturally expressed with high levels of bis-M6P (Mannose 6-Phosphate), designed for increased uptake into muscle cells. Once in the cell, Pombiliti can be properly processed into its most active and mature form to break down glycogen. Opfolda is an enzyme stabilizer designed to stabilize the enzyme in the blood.

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 levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscle cells and glycogen buildup causes muscle damage. Disease severity ranges across a spectrum, with predominant manifestations such as skeletal muscle weakness and progressive respiratory involvement.

This is the second WORLD*Symposium*™ New Treatment Award presented to Amicus. Amicus received the award in 2017 for the first and only oral therapy approved for people living with Fabry disease.

About Pombiliti + Opfolda
Pombiliti + Opfolda, is a two-component therapy that consists of cipaglucosidase alfa-atga, a bis-M6P-enriched rhGAA that 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 reduce loss of enzyme activity in the blood.

U.S. INDICATIONS AND USAGE
POMBILITI in combination with OPFOLDA is indicated for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT).

SAFETY INFORMATION

HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS: Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, POMBILITI should be discontinued immediately and appropriate medical treatment should be initiated. INFUSION-ASSOCIATED REACTIONS (IARs): If severe IARs occur, immediately discontinue POMBILITI and initiate appropriate medical treatment. RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS: Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function, may be at risk of serious exacerbation of their cardiac or respiratory status during POMBILITI infusion. See PI for complete Boxed Warning. CONTRAINDICATION: POMBILITI in combination with Opfolda is contraindicated in pregnancy**. EMBRYO-FETAL TOXICITY:** May cause embryo-fetal harm. Advise females of reproductive potential of the potential risk to a fetus and to use effective contraception during treatment and for at least 60 days after the last dose**. Adverse Reactions:** Most common adverse reactions ≥ 5% are headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia**. Please see full PRESCRIBING INFORMATION, including BOXED WARNING, for POMBILITI (cipaglucosidase alfa-atga) LINK and full PRESCRIBING INFORMATION for OPFOLDA (miglustat) LINK.**

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.

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