



## Amicus Therapeutics Receives Prix Galien U.K. Award for Pombiliti® (cipaglucosidase alfa) + Opfolda® (miglustat) as Best Pharmaceutical Product

June 4, 2024 at 7:00 AM EDT

PRINCETON, N.J., June 04, 2024 (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 Galien Foundation has awarded the 2024 Prix Galien U.K. Award for Best Pharmaceutical Product to Pombiliti® (cipaglucosidase alfa) + Opfolda® (miglustat). The prestigious Prix Galien is awarded to companies recognized for their outstanding advances in the discovery and development of innovative products that improve the overall human condition, and is regarded among the highest accolades for biopharmaceutical and medical technology research.

"It is a great honor for Pombiliti + Opfolda to be awarded the prestigious Prix Galien U.K. Award for Best Pharmaceutical Product," stated Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc. "We believe that this award embodies the dedication and continued success of our team at Amicus, together with patients, physicians and the broader Pompe community, to develop and deliver the first and only two-component therapy for eligible adults living with late-onset Pompe disease. This therapy is the realization of the hard work and efforts of so many individuals dedicated to the mission to make medicines that have a meaningful impact on the lives of people living with rare diseases like Pompe disease. We are grateful to the Galien Foundation for this very important recognition."

### **About the Galien Foundation**

The Galien Foundation fosters, recognises and rewards excellence in scientific innovation to improve the state of human health. Our vision is to be the catalyst for the development of the next generation of innovative treatments and technologies that will impact the state of medical practice and save lives. The Foundation oversees and directs activities in the UK for the Prix Galien, an international awards programme dedicated to progress through innovative medicines development, with chapters in 14 countries, Africa and an inaugural chapter established in India in 2024. The Prix Galien was created in 1970 by Roland Mehl in honour of Galien, the father of medical science and modern pharmacology. Worldwide, the Prix Galien is regarded as the equivalent of the Nobel Prize in biopharmaceutical research.

### **About Pombiliti + Opfolda**

Pombiliti + Opfolda, is a two-component therapy that consists of cipaglucosidase alfa, 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.

### **Important Safety Information**

#### **Pombiliti (cipaglucosidase alfa) Important Safety Information**

**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. **Contraindications:** Life-threatening hypersensitivity to the active substance, or to any of the excipients. Contraindication to miglustat. **Special warnings and precautions for use:** Pombiliti must be used in combination with Opfolda 65 mg hard capsules. **Anaphylaxis and infusion-associated reactions (IARs):** Serious anaphylaxis and IARs have occurred in some patients during infusion and following infusion with Pombiliti. 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.

#### **Opfolda (miglustat) 65 mg hard capsules Important Safety Information**

**Summary of the safety profile:** The most commonly reported adverse reaction only attributable to Opfolda 65 mg was constipation (1.3%). Refer to SmPC for full list. **Contraindications:** Hypersensitivity to the active substance or to any of the excipients. Contraindication to cipaglucosidase alfa. **Special warnings and precautions for use:** Opfolda 65 mg hard capsules must be used in combination with Pombiliti. **Food Interaction:** Patients should fast for 2 hours before and 2 hours after taking Opfolda.

### **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 deteriorating infantile form with significant impact to heart function, to a late-onset form primarily affecting skeletal muscle and progressive respiratory involvement. Late-onset Pompe disease can be severe and debilitating with progressive muscle weakness throughout the body that worsens over time, particularly skeletal muscles and muscles controlling breathing.

### **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](http://www.amicusrx.com), and follow on [X](#) and [LinkedIn](#).

NP-AT-ALL-00020524 May 2024

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