**Prescribing Information: Pombiliti ▼ (cipaglucosidase alfa) 105 mg powder for concentrate for solution for infusion**

**Note:** Please refer to Summary of Product Characteristics (SmPC) before prescribing**.** Pombiliti (cipaglucosidase alfa) must be used in combination with Opfolda (miglustat) 65 mg hard capsules. Because of this, the summary of product characteristics (SmPC) for Opfolda should be consulted in relation to its safety profile before taking Pombiliti. **Presentation:** One vial contains 105 mg of cipaglucosidase alfa. After reconstitution of each vial, the concentrated solution contains 15 mg of cipaglucosidase alfa* per mL. * Human acid α-glucosidase with bis-phosphorylated N-glycans (bis-M6P) is produced in Chinese hamster ovary cells (CHO) by recombinant DNA technology. **Indication:** Pombiliti is a long-term enzyme replacement therapy used in combination with the enzyme stabilizer Opfolda for the treatment of adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency). **Posology and administration:** Treatment should be supervised by a physician experienced in the management of patients with Pompe disease or other inherited metabolic or neuromuscular diseases. Pombiliti must be used in combination with Opfolda 65 mg hard capsules. Posology: The recommended dose of Pombiliti is 20 mg/kg of body weight every other week. The Pombiliti infusion should start 1 hour after taking Opfolda capsules. In the event of infusion delay, the start of infusion should not exceed 3 hours from taking Opfolda. **Switching patients from another enzyme replacement therapy (ERT):** If the patient is switching from another ERT to Pombiliti in combination with Opfolda therapy, the patient can be started with Pombiliti-Opfolda therapy at the next scheduled dosing time (i.e., approximately 2 weeks after the last ERT administration). Patients who have switched from another ERT to Pombiliti in combination with Opfolda therapy should be advised to continue with any premedications used with the previous ERT therapy to minimise infusion-associated reactions (IARs). Depending on tolerability, premedication may be modified. **Missed dose:** If the Pombiliti infusion cannot be started within 3 hours of oral administration of Opfolda, reschedule treatment of Pombiliti and Opfolda at least 24 hours after taking Opfolda. If Pombiliti and Opfolda are both missed, treatment should occur as soon as possible. **Special populations:** Renal and hepatic impairment: The safety and efficacy of Pombiliti in combination with Opfolda therapy have not been evaluated in patients with renal and/or hepatic impairment. No dose adjustment is required in patients with renal impairment. Elderly: There is limited experience with the use of Pombiliti in combination with Opfolda therapy in patients above the age of 65 years old. There is no dose adjustment required in elderly patients. Paediatric population: The safety and efficacy of Pombiliti in combination with Opfolda therapy in paediatric patients less than 18 years old have not yet been established. No data are available. **Method of administration:** Pombiliti should be infused in a stepwise manner. Please consult SmPC for further details. **Contraindications:** Life-threatening hypersensitivity to the active substance, or to any of the excipients. Contraindication to Opfolda. **Special warnings and precautions for use:** Pombiliti must be used in combination with Opfolda 65 mg hard capsules. Anaphylaxis and infusion-associated reactions: Serious anaphylaxis and IARs have occurred in some patients during infusion and following infusion with Pombiliti. 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 if the decision is made to readminister the medicinal product. 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 infusions. 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. Sodium: This medicinal product contains 10.5 mg sodium per vial. This is equivalent of 0.52% of the WHO recommended maximum daily intake of 2 g sodium for an adult. **Interactions:** No interaction studies have been performed. As Pombiliti is a recombinant human protein, it is an unlikely candidate for cytochrome P450 or P-gP mediated interactions with other medicinal products. **Fertility, pregnancy and lactation:** Contraception in females: Reliable contraceptive measures must be used by women of childbearing potential during treatment with Pombiliti in combination with Opfolda, and for 4 weeks after discontinuing treatment. The medicinal product is not recommended in women of childbearing potential not using reliable contraception. Pregnancy: There are no clinical data from the use of Pombiliti in combination with Opfolda in pregnant women. Pombiliti in combination with Opfolda therapy is not recommended during pregnancy. Breast-feeding: It is not known if Pombiliti and Opfolda 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 Opfolda therapy taking into account the benefit of breast-feeding for the child and the benefit of therapy for the woman. Fertility: There are no clinical data on the effects of Pombiliti alone or in combination with Opfolda on fertility. **Undesirable effects:** Very Common (≥1/10): Headache. Common (≥1/100): Anaphylactic reaction, dizziness, tremor, somnolence, dysgeusia, paraesthesia, tachycardia, flushing, hypotension, dyspnoea, cough, diarrhoea, nausea, abdominal pain, flatulence, abdominal distension, vomiting, urticaria, rash, pruritus, hyperhidrosis, muscle spasms, myalgia, arthralgia, muscular weakness, fatigue, pyrexia, chills, chest discomfort, infusion site swelling, pain, peripheral swelling, blood pressure increased. IARs reported in the phase 3 study during or within 2 hours of infusion with Pombiliti included: abdominal distension, chills, pyrexia, dizziness, dysgeusia, dyspnoea, pruritis, rash and flushing. 0.7% of patients experienced anaphylaxis during the phase 3 trial receiving Pombiliti. See SmPC for complete list of adverse reactions. **Legal Category:** POM. **Marketing Authorisation Number:** EU/1/22/1714/001, EU/1/22/1714/002 and EU/1/22/1714/003. **Marketing Authorisation Holder:** Amicus Therapeutics Europe Limited, Block 1, Blanchardstown Corporate Park, Ballycoolin Road, Blanchardstown, Dublin, D15 AKK1, Ireland. **Date of Preparation:** September 2025 **Document Number:** PP-AT-IE-0001-0925. Further information is available from: Amicus Therapeutics UK Ltd. One Globeside, Fieldhouse Lane, Marlow, Buckinghamshire, SL7 1HZ

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions. See section 4.8 of SmPC for how to report adverse reactions.

**Adverse events should be reported.**

**For Ireland**, adverse events should be reported to the Pharmacovigilance Unit at the Health Products Regulatory Authority (HPRA) at www.hpra.ie. Adverse events in Ireland should also be reported to Amicus on 1800936230 or via email to drugsafety@amicusrx.com



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