Prescribing Information: Opfolda (miglustat) 65 mg hard capsules

Note: Please refer to Summary of Product Characteristics (SmPC) before prescribing. Opfolda (miglustat) 65 mg hard capsules must be used in combination with Pombiliti▼(cipaglucosidase alfa). Because of this, the summary of product characteristics (SmPC) for Pombiliti should be consulted in relation to its safety profile before taking Opfolda. Name of product: Opfolda 65mg hard capsules. **Pharmaceutical form:** Each hard capsule contains 65 mg of miglustat. **Indication:** Opfolda is an enzyme stabiliser of Pombiliti long-term enzyme replacement therapy in 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. Opfolda 65 mg hard capsules must be used in combination with Pombiliti. Posology: The recommended dose is to be taken every other week in adults aged 18 years and older and is based on body weight: for patients weighing ≥ 50 kg, the recommended dose is 260 mg (4 capsules of 65 mg) and for patients weighing ≥ 40 kg to < 50 kg, the recommended dose is 195 mg (3 capsules of 65 mg). Opfolda 65 mg hard capsules should be taken orally approximately 1 hour but no more than 3 hours before the start of the Pombiliti infusion. Missed dose: If the Opfolda dose is missed, treatment should occur as soon as possible. If it is not taken, do not start the Pombiliti infusion. Pombiliti infusion can start 1 hour after Opfolda is taken. **Special populations:** Renal and hepatic impairment: The safety and efficacy of Opfolda in combination with Pombiliti therapy have not been evaluated in patients with renal and/or hepatic impairment. No dose adjustment is required in patients with renal or hepatic impairment. Elderly: There is limited experience with the use of Opfolda in combination with Pombiliti 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 Opfolda in combination with Pombiliti therapy in paediatric patients less than 18 years old have not yet been established. No data are available. **Method of administration:** For oral use. Should be swallowed whole on an empty stomach. Patients should fast 2 hours before and 2 hours after taking Opfolda 65 mg hard capsules. During this 4 hour fasting period, water, fat-free (skimmed) cow's milk, and tea or coffee with no cream, sugars, or sweeteners can be consumed. The patient can resume normal eating and drinking 2 hours after taking Opfolda. **Contraindications:** Hypersensitivity to the active substance or to any of the excipients. Contraindication to Pombiliti. **Special warnings and precautions for use:** Opfolda 65 mg hard capsules must be used in combination with Pombiliti. **Interactions:** Food Interaction: Patients should fast for 2 hours before and 2 hours after taking Opfolda. **Fertility, pregnancy, lactation:** Contraception in females: Reliable contraceptive measures must be used by women of childbearing potential during treatment with Opfolda in combination with Pombiliti, 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 Opfolda in combination with Pombiliti in pregnant women. Opfolda in combination with Pombiliti therapy is not recommended during pregnancy. Breast-feeding: It is not known if Opfolda and Pombiliti are secreted in human breast milk. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from Opfolda in combination with Pombiliti 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 Opfolda in combination with Pombiliti therapy on fertility. **Undesirable effects:** Very Common (≥1/10): Headache. Common (≥1/100): Anaphylactic reaction, tremor, dysgeusia, paraesthesia, tachycardia, hypotension, dyspnoea, diarrhoea, nausea, abdominal pain, flatulence, abdominal distension, vomiting, constipation, urticaria, rash, pruritis, hyperhidrosis, muscle spasms, myalgia, arthralgia, muscular weakness, fatigue, pyrexia, chills, peripheral swelling, blood pressure increased. See SmPC for complete list of adverse reactions. **Legal Category:** POM. **Marketing Authorisation Number:** EU/1/23/1737/001 and EU/1/23/1737/002. **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-0002-0925. Further information is available from: Amicus Therapeutics UK Ltd. UK Limited, One Globeside, Fieldhouse Lane, Marlow, Buckinghamshire, SL7 1HZ.

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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