

This guide has been developed by Amicus Therapeutics and is intended for healthcare professionals in GB (England, Scotland and Wales)

YOUR GUIDE TO RECONSTITUTION AND ADMINISTRATION



TREATMENT DAY CHECKLIST



PRODUCTS

- POMBILITI® 105 mg vials** (20 mg/kg body weight administered every other week)
- OPFOLDA® 65 mg capsules** (for patients ≥50 kg, 4 capsules [260 mg total]; for ≥40 kg to <50 kg, 3 capsules [195 mg total])
- Premedications as prescribed
- Adequate contraception as applicable



INFUSION SUPPLIES & EQUIPMENT

- Sterile water** for injection at room temperature of 20°C to 25°C
- Sodium chloride 9 mg/mL (0.9%) solution** for injection at room temperature of 20°C to 25°C – Choose a bag size based on the patient's body weight
- A needle that has a diameter of 18 gauge or less**
- Additional supplies** per institution protocol

Always consult the full SmPC and risk management materials before prescribing and administering POMBILITI® + OPFOLDA®.

INDICATION

Pombiliti® (cipaglicosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency). Opfolda® (miglustat) is an enzyme stabiliser of cipaglicosidase alfa long-term enzyme replacement therapy in adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency).

ADVERSE EVENT REPORTING

▼ 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.

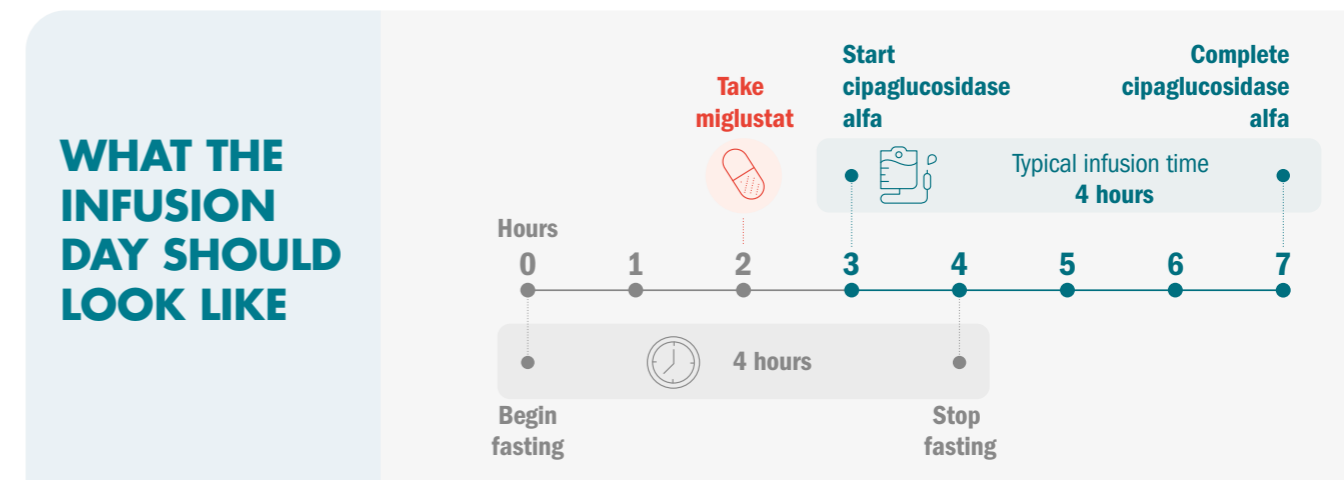
Adverse events should be reported. Reporting forms and information can be found at www.yellowcard.mhra.gov.uk
Adverse events should also be reported to Amicus on 08082346864 or via email to drugsafety@amicusrx.com

Full Prescribing Information for Pombiliti® and Opfolda® can be found at the end of this document.

TIMELINE TO ADMINISTRATION^{1,2}

POMBILITI® must be used in combination with OPFOLDA®

The use of OPFOLDA® with any other rhGAA enzyme replacement therapy has not been studied.



If the patient is switching from another enzyme replacement therapy (ERT), treatment with POMBILITI® + OPFOLDA® can be started at the next scheduled dosing time—approximately 2 weeks after the last ERT administration. Patients who have switched from another ERT to POMBILITI® + OPFOLDA® therapy should be advised to continue with any premedications used with the previous therapy to minimize infusion-associated reactions (IARs).



Note that dosing for both POMBILITI® + OPFOLDA® is based on weight. Double-check the following:

- The patient has taken the OPFOLDA® capsules approximately 1 hour before the infusion is due to begin
- They've been fasting for 2 hours before and 2 hours after taking OPFOLDA®

Taking OPFOLDA® :

- Capsules should be swallowed whole and taken on an empty stomach
- The patient should **fast 2 hours before and 2 hours after taking OPFOLDA®**
 - 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.
- Capsules should be taken approximately **1 hour before the start of POMBILITI® infusion**
 - In the event of infusion delay, the start of infusion should not exceed 3 hours from the oral administration of OPFOLDA®
- Two hours after taking OPFOLDA®, the patient can resume normal eating and drinking

rhGAA, recombinant human acid alpha-glucosidase.

Pombiliti® (cipagluco­sidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency).

HELPFUL INFORMATION FOR DOSING^{1,2}

Dosing for both POMBILITI® + OPFOLDA® is based on body weight

If the patient's weight has changed since their last infusion, or if you haven't administered POMBILITI® + OPFOLDA® to the patient before, you will need to weigh the patient.

Calculating the dose

POMBILITI® is administered to the patient by intravenous infusion every other week in combination with the oral medication **OPFOLDA®**.

Weight-based dosing

Recommended dosage:
 20 mg/kg of total body weight administered once every other week as an intravenous infusion

OPFOLDA® capsules of 65 mg are administered every other week in combination with the intravenous infusion **POMBILITI®**.

Weight-based dosing

Recommended dosage:
 • ≥ 40 kg to < 50 kg = 3 capsules
 • ≥ 50 kg = 4 capsules





	Calculation	Example
Dose	Patient's body weight (kg) x dose (20 mg/kg)	65 kg x 20 mg/kg = 1300 mg total dose
Number of vials	Patient's dose (in mg) divided by 105 (mg/vial)	1300/105 mg per vial = 12.38 vials
	Round up to the nearest whole vial	12.38 vials → 13 vials
Calculate extraction volume	Number of full vials x 7.0 mL/bottle extraction volume	12 vials x 7.0 mL = 84 mL
		0.38 vial x 7.0 mL = 2.7 mL
		84 mL + 2.7 mL = 86.7 mL extraction volume

PREPARING FOR RECONSTITUTION¹



Before POMBILITI® can be administered to the patient, it must be reconstituted

- Once you know how many vials you'll be using, take them out of the refrigerator and let them stand for about 30 minutes to reach room temperature of 20°C to 25°C
- Each vial of Pombiliti® is for single use only
- Use aseptic technique

Items needed for reconstitution and dilution:

	POMBILITI® 105 mg vials		Sterile water for injection at room temperature of 20°C to 25°C
	Sodium chloride 9 mg/mL (0.9%) solution for injection at room temperature of 20°C to 25°C – Choose a bag size based on the patient's body weight		A needle with a diameter of 18 gauge or less

Inspect each vial carefully.

	Clear to opalescent, colourless to slightly yellow, and appears almost free of particles, but may contain white-to-translucent particles.		Do not use if foreign particles are observed or if the solution is discoloured.
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Pombiliti® (cipaglusidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency).

RECONSTITUTING¹

Reconstituting the lyophilised powder:



1.

Remove vials from the refrigerator (2°C to 8°C) and allow to come to room temperature (ie, approximately 30 minutes at 20°C to 25°C).



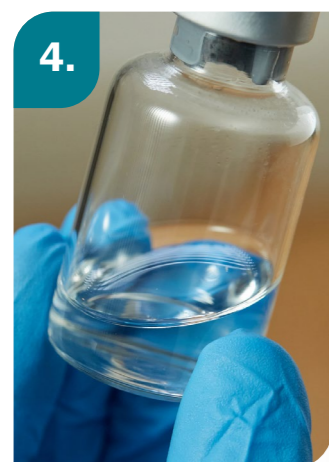
2.

Reconstitute each vial by slowly adding 7.2 mL sterile water for injection dropwise down onto the lyophilised powder. Avoid forceful impact of sterile water for injection on the lyophilised powder and avoid foaming.



3.

Tilt and roll each vial gently to dissolve the powder. Do not invert, swirl or shake. Reconstitution of the lyophilised powder typically takes 2 minutes.



4.

Perform an inspection of the reconstituted vials for particulate matter and discoloration. The reconstituted volume appears as a clear to opalescent, colourless to slightly yellow solution, free of foreign particles, and practically free of particles in the form of white to translucent particles.

- Do not use if upon immediate inspection foreign particles other than those described above are observed, or if the reconstituted solution is discoloured
- Each reconstituted vial should yield a concentration of 15 mg/mL with an extractable volume of 7.0 mL

5. Repeat the above steps for the number of vials needed for dilution.

DILUTING¹

Diluting the solution:



1.

Select an intravenous (IV) bag with sufficient volume to achieve a final target concentration range of 0.5 mg/mL to 4 mg/mL for the diluted POMBILITI® solution for IV infusion.



2.

Remove airspace within the infusion bag. Remove an equal volume of sodium chloride 9 mg/mL (0.9%) solution for injection that will be replaced by the total volume (mL) of reconstituted POMBILITI®.



3.

The reconstituted volume allows accurate withdrawal of 7.0 mL (equal to 105 mg) from each vial. Slowly withdraw the reconstituted solution from the vials until the patient's dose is obtained. Avoid foaming in the syringe. Discard any remaining reconstituted solution in the last vial.



4.

Slowly inject reconstituted POMBILITI® directly into the sodium chloride 9 mg/mL (0.9%) solution for injection bag. Do not add directly into the air space that may remain within the infusion bag.



5.

Gently invert or massage the bag to mix the diluted solution.

- Do not shake or excessively agitate the bag for infusion
- Do not use a pneumatic tube to transport the infusion bag



- **POMBILITI® should be administered as stated in the Summary of Product Characteristics**
- **The infusion solution should be administered as close to after dilution preparation as possible at room temperature**
- **An intravenous administration set should be used with an inline low protein binding 0.2 micron filter. If the intravenous line blocks during infusion, change the filter**
- **If immediate use is not possible, the reconstituted solution may be stored for up to 24 hours under refrigeration at 2°-8° C**

Pombiliti® (cipaglusosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency).

ADMINISTERING THE INFUSION¹

Now you're ready to begin the infusion



The infusion will take approximately 4 hours.



Treatment should be supervised by a physician experienced in the management of patients with Pompe disease or other inherited metabolic or neuromuscular diseases.

Recommended Infusion Volumes and Rates

Patient Weight Range (kg)	Total Infusion Volume (mL)	Step 1	Step 2	Step 3	Step 4
		1 mg/kg/hr	3 mg/kg/hr	5 mg/kg/hr	7 mg/kg/hr
		Infusion rate in mL/hr			
40–50	250	13	38	63	88
50.1–60	300	15	45	75	105
60.1–100	500	25	75	125	175
100.1–120	600	30	90	150	210
120.1–140	700	35	105	175	245

- The infusion solution should be administered at room temperature.
- Total volume of infusion is determined by body weight and typically administered over approximately 4 hours, if tolerated.
- Infusion of POMBILITI[®] should start approximately **1 hour after oral administration of OPFOLDA[®]**.
- In the event of infusion delay, the **start of infusion should not exceed 3 hours from the oral administration of OPFOLDA[®]**.
- Infusion should be administered in a stepwise manner.
- The initial infusion rate of 1 mg per kg per hour is recommended.
- The infusion rate may be gradually increased by 2 mg per kg per hour every 30 minutes, if there are no signs of infusion-associated allergic reactions (IARs), until a maximum rate of 7 mg per kg per hour is reached.

The infusion rate may be slowed or temporarily stopped in the event of mild-to-moderate IARs.
In the event of severe allergic, anaphylaxis, serious or severe IARs, immediately stop the infusion and initiate appropriate medical treatment.

Disposal

Any unused medicinal product or waste material should be disposed of in accordance with local requirements.

SAFETY¹⁻³

Always consult the full SmPC and risk management materials before prescribing and administering POMBILITI[®] + OPFOLDA[®].

Contraindications

- POMBILITI** is contraindicated in patients with life-threatening hypersensitivity to the active substance, or to any of the excipients listed in the SmPC, when rechallenge was unsuccessful
- OPFOLDA** is contraindicated in patients with a hypersensitivity to the active substance, or to any of the excipients as listed in the SmPC

Immunogenicity

- Overall, there was **no apparent association between immunogenicity** and safety, PK, or PD effects
- Patients should be monitored** for signs and symptoms of systemic immune complex-related reactions

Most commonly reported adverse reactions (≥ 1/10)

- POMBILITI: The most commonly reported adverse reactions** only attributable to cipaglucosidase alfa 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%).
- OPFOLDA: The most commonly reported adverse reactions** only attributable to miglustat 65 mg was constipation (1.3%).

Infusion-associated reactions (IARs)

- In the Phase 3 study, the following IARs were reported during or within 2 hours after completion of the POMBILITI infusion: abdominal distension, chills, pyrexia, dizziness, dysgeusia, dyspnoea, pruritus, rash, and flushing
- 0.7% of patients receiving POMBILITI + OPFOLDA experienced a serious adverse reaction of anaphylaxis (characterised by generalised pruritus, dyspnoea, and hypotension) during the Phase 3 trial. 1.3% of patients receiving POMBILITI + OPFOLDA discontinued treatment due to IARs (anaphylaxis and chills). Most IARs were mild or moderate in severity and transient in nature

Risk management information

FERTILITY, PREGNANCY AND LACTATION

- Reliable contraceptive measures must be used by women of childbearing potential during and for 4 weeks after stopping treatment. POMBILITI + OPFOLDA is not recommended for use in women of childbearing potential not using reliable contraception.
- POMBILITI + OPFOLDA is not recommended for use during pregnancy.
- It is not known whether POMBILITI + OPFOLDA are excreted in human breast milk. A risk to new-borns/infants cannot be excluded. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from POMBILITI + OPFOLDA taking into account the benefit of breast-feeding for the child and the benefit of therapy for the woman.
- There are no clinical data on the effects of POMBILITI + OPFOLDA on fertility.
- Please consult the SmPC.

PD, pharmacodynamics; PK, pharmacokinetics.

PRESCRIBING INFORMATION

Pombiliti® ▼ (cipaglusosidase alfa) 105 mg powder for concentrate for solution for infusion

Note: Please refer to Summary of Product Characteristics (SmPC) before prescribing. Pombiliti (cipaglusosidase 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 cipaglusosidase alfa. After reconstitution of each vial, the concentrated solution contains 15 mg of cipaglusosidase 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 on fertility. **Undesirable effects: Very Common ($\geq 1/10$):** Headache. **Common ($\geq 1/100$):** Anaphylactic reaction, dizziness, tremor, somnolence, dysgeusia, tachycardia, flushing, dyspnoea, cough, diarrhoea, nausea, abdominal pain, flatulence, abdominal distension, vomiting, urticaria, rash, pruritus, hyperhidrosis, muscle spasms, myalgia, muscular weakness, fatigue, pyrexia, chills, chest discomfort, infusion site swelling, pain, 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, pruritus, rash and flushing. 0.7% of patients experienced anaphylaxis during the phase 3 trial receiving Pombiliti. See SmPC for complete list of adverse reactions. **List price:** 1 vial £987.00 **Legal Category:** POM. **Marketing Authorisation Number:** PLGB 25823/0003. **Marketing Authorisation Holder:** Amicus Therapeutics UK Limited, One Globeside, Fieldhouse Lane, Marlow, Buckinghamshire, SL7 1HZ. **Date of Preparation:** August 2023 **Document Number:** PP-AT-UK-0007-0823. Further information is available from: Amicus Therapeutics UK Ltd.

ADVERSE EVENTS SHOULD BE REPORTED

▼ 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.

For the UK (including Northern Ireland), reporting forms and information can be found at www.yellowcard.mhra.gov.uk. Adverse events should also be reported to Amicus on 08082346864 or via email to drugsafety@amicusrx.com

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 ▼ (cipaglusosidase 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 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 ($\geq 1/10$):** Headache. **Common ($\geq 1/100$):** Tremor, dysgeusia, tachycardia, dyspnoea, diarrhoea, nausea, abdominal pain, flatulence, abdominal distension, vomiting, constipation, urticaria, rash, pruritus, hyperhidrosis, muscle spasms, myalgia, muscular weakness, fatigue, pyrexia, chills, blood pressure increased. See SmPC for complete list of adverse reactions. **List price:** 24 capsule bottle £700.14. **Legal Category:** POM. **Marketing Authorisation Number:** PLGB 25823/0004. **Marketing Authorisation Holder:** Amicus Therapeutics UK Limited, One Globeside, Fieldhouse Lane, Marlow, Buckinghamshire, SL7 1HZ. **Date of Preparation:** August 2023 **Document Number:** PP-AT-UK-0008-0823. Further information is available from: Amicus Therapeutics UK Ltd.

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REFERENCES

- Amicus Therapeutics (2023). Pombiliti 105 mg powder for concentrate for solution for infusion SmPC.
- Amicus Therapeutics (2023). Opfolda 65 mg hard capsules SmPC.
- Schofer B, Roberts M, Byrne BJ, et al. Safety and efficacy of cipaglusosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial. *Lancet Neurol.* 2021;20(12):1027-1037. doi:10.1016/S1474-4422(21)00331-8

 **Pombiliti**[®] +  **Opfolda**[®]
(cipaglucosidase alfa) (miglustat) 65 mg capsules

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