

GUIDE TO STARTING A PATIENT ON TREATMENT

Treatment Day Checklist and Frequently Asked Questions for Healthcare Professionals in the UK and Ireland

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

Find a treatment day checklist and answers to common questions about the administration of POMBILITI® (cicapglucosidase alfa) + OPFOLDA® (miglustat), as well as information about additional resources available.

INDICATION

Pombiliti® (cicapglucosidase 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 cicapglucosidase alfa long-term enzyme replacement therapy in adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency).

ADVERSE EVENT REPORTING

Adverse events should be reported.

In the UK, reporting forms and information can be found at yellowcard.mhra.gov.uk. Adverse events should also be reported to Amicus on 0808 234 6864 or via email to drugsafety@amicusrx.com.

In Ireland, reporting forms and information can be found at www.hpra.ie. Adverse events in Ireland should also be reported to Amicus on 1800936230 or via email to drugsafety@amicusrx.com.

▼ 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.

TREATMENT DAY CHECKLIST

Key Dosing Considerations

- Confirm appropriate fasting 2 hours before and after the patient has taken OPFOLDA®¹
- Check patient weight to confirm sufficient POMBILITI® vial supply and OPFOLDA® capsule supply^{1,2}
- Inspect each vial carefully and do not use it if the content is discolored, the closure is damaged, and/or the overseal button is missing or has been removed²
- Refer to the Guide to Reconstitution and Administration
- Review the full Prescribing Information for POMBILITI® and full Prescribing Information for OPFOLDA®

Products

- POMBILITI® (cipaglucosidase alfa) 105-mg vials**
(20 mg/kg body weight administered as an infusion every other week)²
- OPFOLDA® (miglustat) capsules**
(for patients ≥ 50 kg, 4 capsules of 65 mg taken orally every other week [260 mg total];
for ≥ 40 kg to < 50 kg, 3 capsules [195 mg total] taken orally every other week)¹
- Premedications as prescribed
- Adequate contraception as applicable

Infusion Supplies & Equipment

- Sterile water** for injection at room temperature of 20 °C to 25 °C²
- 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 weight²
- A needle that has a diameter of 18 gauge or less**
 - Do not use filter needles²
- Infusion pump, tubing, and filter²**

FREQUENTLY ASKED QUESTIONS

About POMBILITI® + OPFOLDA®



Who is POMBILITI® + OPFOLDA® appropriate for?



POMBILITI® (cicapglucosidase 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 cicapglucosidase alfa long-term enzyme replacement therapy in adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency).



How does POMBILITI® work?



POMBILITI® is an enzyme replacement therapy that degrades excess lysosomal glycogen. POMBILITI® contains bis-M6P—a type of sugar that can bind to receptors on muscle cells. Bis-M6P allows POMBILITI® to be internalized by muscle cells and transported to lysosomes. Once inside the lysosome, POMBILITI® undergoes modifications (proteolytic cleavage and N-glycan trimming), and then exerts enzymatic activity in cleaving glycogen.²



How does OPFOLDA® work?



OPFOLDA® binds with, stabilizes, and reduces inactivation of POMBILITI® in the blood after infusion. The bound OPFOLDA® is dissociated from POMBILITI® after it is internalised and transported into lysosomes. OPFOLDA® alone has no pharmacological activity in cleaving glycogen.¹



Can a patient switch from another ERT to POMBILITI® + OPFOLDA® right away?



A patient can be started on POMBILITI® + OPFOLDA® at the next scheduled dosing if switching from another ERT (ie, 2 weeks after the last ERT administration).

Patients who have switched from another ERT to POMBILITI® + OPFOLDA® should be advised to continue with any premedications used with the previous ERT therapy to minimise infusion-related allergic reactions (IARs). Depending on tolerability, premedications may be modified. Premedication and/or treatment during infusion with corticosteroids, antihistamines, and antipyretics may be administered to assist with signs and symptoms related to IARs.²



What is the purpose of providing OPFOLDA® prior to starting infusion with POMBILITI®?



OPFOLDA® should be taken approximately 1 hour prior to POMBILITI® so it has time to be absorbed. Given time to absorb, it can then be available to stabilize POMBILITI® in the unfavorable conditions of the blood during infusion to reduce enzyme loss while in circulation.¹



Can either medication be provided without the other?



POMBILITI® must be taken in combination with OPFOLDA® (65 mg). Because POMBILITI® + OPFOLDA® were designed to work exclusively with each other, they must be taken at the right time under the right circumstances.

FREQUENTLY ASKED QUESTIONS

Dosing and Administration

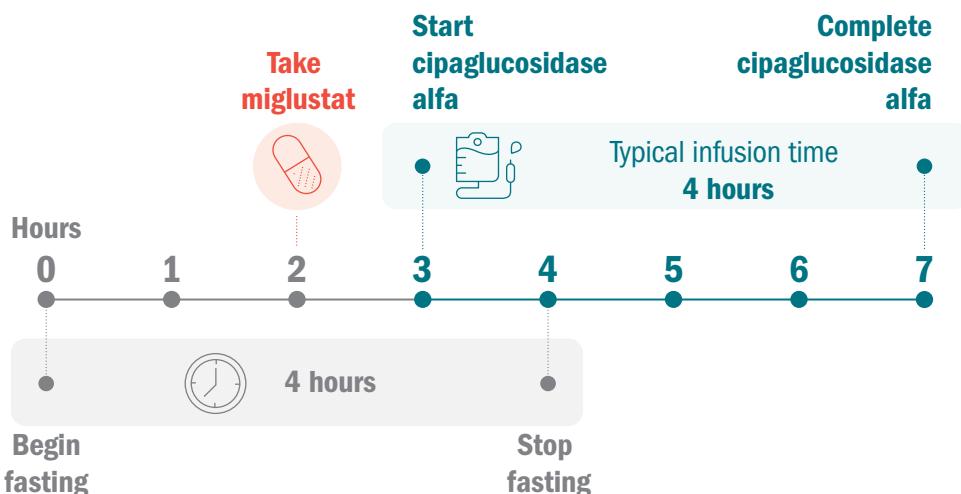


On average, how long does it take to administer POMBILITI® + OPFOLDA®?



The entire process takes about 7 hours, from the beginning of the fast until the end of the infusion. The infusion itself takes about 4 hours. Please see below for a detailed timeline.²

What the infusion day should look like



How long should a patient fast prior to taking OPFOLDA®?



Patients are required to fast for 2 hours prior to and 2 hours after taking OPFOLDA®. OPFOLDA® should be taken on an empty stomach. Capsules should be swallowed whole. During the fasting period, water, fat-free (skimmed) cow's milk, and tea or coffee with no cream, sugars, or sweeteners can be consumed. Please refer to the timeline above.¹



How do I calculate the number of vials of POMBILITI® needed for my patient?



The formula used for dosing POMBILITI® is as follows:

Patient body weight (kg) x 20 mg/kg of body weight = Total dose of POMBILITI® (mg) administered every other week/105 mg per vial = Number of vials needed

Example: 85 kg x 20 mg/kg = 1700 mg/105 mg per vial = 16.19 vials > 17 vials to reconstitute.²

FREQUENTLY ASKED QUESTIONS

Dosing and Administration



What is the infusion rate for POMBILITI®?



An initial infusion rate of 1 mg/kg/hour is recommended. This infusion rate may be gradually increased by 2 mg/kg/hour every 30 minutes if there are no signs of infusion-associated allergic reactions (IARs) until a maximum rate of 7 mg/kg/hour is reached. Then, maintain the infusion rate at 7 mg/kg/hour until the infusion is complete. The approximate total infusion duration is 4 hours. Vital signs should be obtained at the end of each step.

The infusion rate may be slowed or temporarily stopped in the event of mild to moderate IARs. In the event of severe allergic reaction or anaphylaxis, immediately stop the infusion, and initiate appropriate medical treatment.²



What happens if there is a greater than 3-hour delay in starting POMBILITI® after taking OPFOLDA®?



If the POMBILITI® infusion cannot be started within 3 hours of oral administration of OPFOLDA®, reschedule treatment of POMBILITI® + OPFOLDA® at least 24 hours after OPFOLDA® was last taken.²



How is POMBILITI® supplied?



POMBILITI® is supplied as a sterile, nonpyrogenic, white to slightly yellowish lyophilised powder for reconstitution with sterile water for injection to yield a concentration of 15 mg/mL, then further diluted with 0.9% sodium chloride for injection for intravenous infusion.

Single-use vials are available in 105-mg dosage only.²

FREQUENTLY ASKED QUESTIONS

Dosing and Administration (continued)



How is OPFOLDA® supplied?



OPFOLDA® capsules are supplied as 65 mg of miglustat, with a grey opaque cap and a white opaque body, printed with "AT2221" in black ink on the body.¹



What are the key steps to reconstituting POMBILITI®?



Use aseptic technique during preparation. Reconstitute and dilute POMBILITI® in the following manner:

Reconstitute the Lyophilised Powder

- Determine the number of POMBILITI® vials to be reconstituted based on the calculated dose (based on patient's actual body weight in kg).
- Remove vials from the refrigerator and set aside for approximately 30 minutes to allow vials to come to room temperature.
- Reconstitute each vial by slowly injecting 7.2 mL of sterile water for injection, down the inside wall of each vial to avoid foaming. Avoid forceful impact of sterile water for injection on the lyophilised powder and avoid foaming.
- Roll and tilt each vial to allow the lyophilised powder to dissolve completely which typically takes 2 minutes. Each vial will yield a concentration of 15 mg/mL. Do not invert, swirl, or shake.
- Visually inspect the reconstituted solution for particulate matter and discoloration. The reconstituted solution appears as a clear to opalescent, colorless to slightly yellow solution, free of foreign particles and practically free of white to translucent particles. Discard if foreign matter is observed or the solution is discolored.
- Repeat above steps for the number of vials needed for dilution.

Storage of the Reconstituted Solution

- If the reconstituted POMBILITI® vials are not used immediately, store refrigerated at 2°C to 8°C for up to 24 hours. Do not freeze.

Dilute the Reconstituted Solution

- Remove airspace within an infusion bag of 0.9% (9 mg/mL) sodium chloride solution for injections. Remove an equal volume of 0.9% Sodium Chloride Injection from the bag that will be replaced by the total volume (mL) of reconstituted POMBILITI® (see Table 1 for the recommended total infusion volume based on the patient's weight).
- Slowly withdraw 7 mL of reconstituted solution from each of the vials until the patient's dose is obtained. Discard any remaining reconstituted solution in the last vial.
- Add the reconstituted solution slowly and directly into the infusion bag.
- To prevent foaming, gently invert infusion bag to mix the solution and avoid vigorous shaking or agitation. After dilution, the solution will have a final concentration of 0.5 to 4 mg/mL of cipaglucosidase alfa. Do not use a pneumatic tube to transport the infusion bag.
- Repeat steps on remaining infusion bag(s) to achieve the total volume (mL) of reconstituted cipaglucosidase alfa required for the patient's dose.
- Administer the diluted solution at room temperature without delay.²

FREQUENTLY ASKED QUESTIONS

Dosing and Administration (continued)



How should I store POMBILITI®?



Please store in a refrigerator (2°C - 8°C) and in original packaging to protect from light.²



Do I need to let POMBILITI® come to room temperature prior to reconstitution or infusion?



Yes, the infusion should be administered at room temperature. Please remove vials from the refrigerator approximately 30 minutes prior to reconstitution.²



How long will a typical infusion last?



The typical infusion is administered over approximately 4 hours. The total volume of the infusion is determined by the patient's body weight.²

Dietary Requirements



What can my patient eat and drink during the fast?



During this 4-hour fasting period patients can consume water, fat-free (skimmed) cow's milk, and tea or coffee with no cream, sugars, or sweeteners.¹



What can my patient eat and drink during the infusion?



Patients can resume normal eating and drinking 2 hours after taking OPFOLDA®. Typically this would be about an hour after the infusion starts.¹



What if a patient does not properly fast prior to taking OPFOLDA®?



Patients should not take OPFOLDA® unless they have adhered to the fasting guidelines for 2 hours prior to administration.¹

FREQUENTLY ASKED QUESTIONS

Potential Adverse Events



What are the side effects of POMBILITI® + OPFOLDA®?



The most common adverse reaction in patients treated with POMBILITI® and OPFOLDA® ($\geq 1/10$) was headache. The following adverse reactions were common ($\geq 1/100$ to $< 1/10$): 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 and peripheral swelling.

The most commonly reported adverse reactions only attributable to POMBILITI® were 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%).

The most commonly reported adverse reaction only attributable to OPFOLDA® was constipation (1.3%).



Are there any premedications required?



Please consult the prescriber to coordinate appropriate premedications.



Patients who have switched from another ERT to POMBILITI® + OPFOLDA® should be advised to continue with any premedications used with the previous ERT therapy to minimize IARs. Depending on tolerability, premedications may be modified. Premedication and/or treatment during infusion with corticosteroids, antihistamines, and antipyretics may be administered to assist with signs and symptoms related to IARs.²



Are there any considerations when providing this medication to special populations such as children or elderly patients?



This medicine should not be given to patients under the age of 18 years old. The safety and effectiveness have not been established in children. There is limited experience in patients above the age of 65 years old but there is currently no evidence for special consideration.²



Are there any contraindications with POMBILITI® + OPFOLDA®?



POMBILITI® in combination with OPFOLDA® is contraindicated in individuals with a hypersensitivity to the active substance or to any of the excipients listed in the full Summary of Product Characteristics. Treatment is also not recommended during pregnancy.²



Are POMBILITI® + OPFOLDA® contraindicated in pregnancy?



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.

FREQUENTLY ASKED QUESTIONS

Potential Adverse Events

(continued)



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® in combination with OPFOLDA® on fertility. Please consult the SmPC for further information.

Infusion-Associated Reactions



What do I do in the event of an infusion-associated reaction?



If severe infusion-associated reactions occur, immediately pause the POMBILITI® infusion, initiate appropriate medical treatment, and carefully assess the benefits and risks of readministering POMBILITI® following severe IARs. Patients may be rechallenged using slower infusion rates. Once a patient tolerates the infusion, the infusion rate may be increased to reach the recommended infusion rate. If mild or moderate IARs occur regardless of pretreatment, decreasing the infusion rate or temporarily stopping the infusion may ameliorate the symptoms.²



What do I do in the event of a hypersensitivity reaction including anaphylaxis?



If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, POMBILITI® should be paused immediately, and appropriate medical treatment should be initiated. The risks and benefits of readministering POMBILITI® following severe hypersensitivity reaction (including anaphylaxis) should be considered. Patients may be rechallenged using slower infusion rates. In patients with severe hypersensitivity reaction, desensitization measures to POMBILITI® may be considered. If the decision is made to readminister POMBILITI®, ensure the patient tolerates the infusion. If the patient tolerates the infusion, the dosage (dose and/or the rate) may be increased to reach the approved recommended dosage. If a mild or moderate hypersensitivity reaction occurs, the infusion rate may be slowed or temporarily stopped.

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

STORAGE

How to Store POMBILITI®²

POMBILITI® is supplied as a sterile, nonpyrogenic, white to slightly yellowish lyophilised powder for reconstitution with sterile water for injection to yield a concentration of 15 mg/mL; then further diluted with 0.9% sodium chloride for injection for intravenous infusion. Single use vials are available in 105 mg dosage only.

- Store at 2 °C to 8 °C
- Do not freeze
- Store in the original packaging to protect from light

Storage for reconstituted and diluted solution

- Do not freeze the reconstituted vial or the diluted POMBILITI® solution in the infusion bag for infusion

Infusion Preparation	Infusion-use Stability	
	Refrigerated Storage 2 °C to 8 °C	Room Temperature Storage 20 °C to 25 °C
Once POMBILITI® vial is reconstituted with sterile water for injection	24 hours	Not recommended
Once reconstituted vial is diluted with sodium chloride 9 mg/mL (0.9%) solution for injection in the infusion bag	24 hours	6 hours

How to Store OPFOLDA®¹

- OPFOLDA® is supplied as a hard gelatin capsule containing 65 mg of miglustat
- Store at 20 °C to 25 °C. Excursions are permitted between 15 °C to 30 °C
- Do not use if inner seal is missing or broken
- Keep out of reach of children
- Store in the original container or equivalent to protect from light

PRESCRIBING INFORMATION



[Click here or scan the QR code for UK POMBILITI prescribing information](#)



ADVERSE EVENTS SHOULD BE REPORTED

In the UK, reporting forms and information can be found at yellowcard.mhra.gov.uk. Adverse events should also be reported to Amicus on 0808 234 6864 or via email to drugsafety@amicusrx.com.

[Click here or scan the QR code for Ireland POMBILITI prescribing information](#)



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▼ 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.



(miglustat) 65 mg capsules

[Click here or scan the QR code for UK OPFOLDA prescribing information](#)



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REFERENCES

1. OPFOLDA® Summary of Product Characteristics;
2. POMBILITI® Summary of Product Characteristics.

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