

Home infusion guide for healthcare professionals

VERSION 1.0

Home infusion guide for healthcare professionals v1.0
has been approved by the HPRA March 2026

Healthcare Professionals involved in the prescribing and administration of Pombiliti (cipaglucosidase alfa) must read and understand the information contained within this pack.

For complete safety information please refer to the Summary of Product Characteristics (SmPC) for cipaglucosidase alfa, available on the European Medicines Agency (EMA) website:
<https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>

▼ 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 using the HPRA Website: www.hpra.ie.

Adverse reactions should also be reported to Amicus Medical Information on: Tel: 1800 936 230;
email: drugsafety@amicusrx.com

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1

CONTENT AND OBJECTIVES

CipaglucoSIDase alfa infusion therapy, in combination with miglustat, is available for treatment of patients with Pompe disease. To improve convenience and quality of life, the intravenous treatment can be administered in the patient's home if specific requirements can be fulfilled.

About this guide

The purpose of this guide for physicians and healthcare professionals is to minimise the risk for medication errors when cipaglucoSIDase alfa is administered at home, and to provide clear guidance on the following:

- **Which patients may be eligible for home infusion, and how to organize home infusions, including premedication and emergency treatment of infusion reactions;**
- **Preparation, reconstitution, and administration of the product at home;**
- **Recognition and management of adverse drug reactions, including infusion associated reactions (IARs), and possible actions for the management of adverse drug reactions when symptoms occur;**
- **Recognition and management of Medication Errors.**

The home infusion takes place under the responsibility of the treating physician. Distribution of the patient's educational material should only be executed if the treating physician decides that the patient is eligible for home infusion treatment.

The processes presented in this document serve as overall guidance but are subject to local medical practice and national rules and regulations.

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ASSESSING ELIGIBILITY FOR HOME INFUSION

The decision to have a patient move to home infusion should be made after evaluation and upon recommendation by the treating physician. A patient's underlying co-morbidities and ability to adhere to the home infusion requirements need to be taken into account when evaluating the patient for eligibility to receive home infusion. Before making any arrangements, the treating physician must determine if the patient fulfils the following primary criteria for a home infusion:

(SEE ALSO THE PRESCRIBING INFORMATION AVAILABLE ON EMA WEBSITE:
<https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>)

- The patient must be tolerating their infusions well, and have no history of moderate or severe IARs for a few months.
- The patient must have received cipaglusosidase alfa infusions supervised by a physician with expertise in management of Pompe disease patients for a few months in a hospital or clinic setting. Documentation of a pattern of well-tolerated infusions is a prerequisite for the initiation of home infusion.
- The patient must be considered medically stable. A comprehensive evaluation must be completed before the initiation of home infusion.
- The patient must have a history of adherence to the prescribed dosing schedule and miglustat fasting requirements.
- The patient must be willing and able to comply with home infusion procedures.
- The patient must have no ongoing concurrent condition that, in the opinion of the physician, may affect patient's ability to tolerate the infusion.
- Home infusion infrastructure, resources, and procedures, including training, must be established and available to the healthcare professional. The healthcare professional should be always available during the home infusion and for a specified time after infusion, depending on patient's tolerance prior to starting home infusion.

3

MEDICAL EXAMINATION OF THE PATIENT BEFORE ADMINISTERING THE HOME INFUSION

Before each cipaglucoSIDase alfa infusion is administered, the patient's fitness to be infused should be established. The patient should be in good general condition and well-being according to their individual disease status.

The presence of acute as well as febrile illness should be excluded. The patient should be questioned as to whether any adverse reactions occurred in association with the previous infusion (i.e., after the infusion healthcare professional/nurse left the patient's home).

Blood pressure, pulse, respiration rate and body temperature should be checked regularly at the beginning and also during the infusion, particularly before any increase in the infusion rate.

In case of intercurrent illness or newly reported adverse reactions in association with previous infusion, consultation is required with the treating physician before starting the infusion.

3.1

Pre-treatment and emergency treatment

- **Premedication with oral antihistamine, antipyretics, and/or corticosteroids may be administered to assist with signs and symptoms related to mild IARs. Appropriate pre-treatment should be provided based on the patient-specific prescription.**
Treatment administered in the hospital or other medical setting should not be altered in the home setting unless medically warranted at the discretion of the treating physician.
- **Medications must be available to respond to an emergency situation, if necessary.**
Proper education on the use of emergency medications must be provided by the treating physician to the home infusion healthcare professional/nurse.
- **In the event the patient experiences an IAR, appropriate management including reduction of the infusion rate, temporary interruption of the infusion, symptomatic treatment with oral antihistamine, or antipyretics, and appropriate resuscitation measures should be considered.** Section 6 provides a guideline for management of IARs; however, the list of IAR symptoms is not comprehensive and is meant to serve as a guideline to the infusion healthcare professionals/nurses for the initial management of IARs, including anaphylaxis. It is at the treating

physician's discretion to follow the guidelines or implement the treatment protocol that their centre is accustomed to following.

- **In the event the patient experiences anaphylaxis or a severe allergic reaction** during or shortly after the infusion, **the infusion should be immediately stopped 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. If the patient continues therapy, their next infusions **must occur in a hospital** or other medical setting equipped to deal with such medical emergencies.

3.2

The Infusion diary

- The infusion diary serves as a **means of communication for all involved** in administering cipaglucoisidase alfa in combination with miglustat in the home-setting.
- The infusion healthcare professional/nurse **records the findings and actions** from the initial infusion and all relevant information from subsequent infusions in the infusion diary.
- **An emergency contact list must be completed** and available at home in the infusion diary for the patient and/or caregiver(s) and the infusion healthcare professional/nurse.
- The infusion diary must be **kept at the patient's home** and is updated by the infusion healthcare professional/nurse and/or patient/caregiver(s) each time cipaglucoisidase alfa is administered.
- The patient/caregiver **must take the infusion diary to the hospital/clinic** at each appointment and bring it home afterwards.
- In the infusion diary, **the treating physician clearly states the dose, the required reconstituted volume, infusion rate, as well as any changes.**
- The infusion diary can be used to record any IARs, including allergic-type hypersensitivity reactions before, during or after the infusion.
- The treating physician clearly states what has to be done, which **pre-medications should be administered**, and **which medications are to be administered in the event of a serious IAR** in line with current medical standards for emergency treatment.
- The **contact details of the treating physician** and the country-specific national emergency number are documented in the infusion diary.

4

HOME INFUSION PROCEDURE

The treating physician is responsible for the organisation of the home infusion and needs to agree upon the home infusion procedure and ensure the home infusion healthcare professional/nurse is appropriately qualified.

The infusion healthcare professional/nurse carries out the entire procedure for the infusions at the patient's home. The infusion healthcare professional/nurse is available at all times during the home infusion and for a specified time after infusion, depending on patient's tolerance prior to starting home infusion.

The preparation/administration procedures described in **Section 5** of this document must be adhered to, and each administration of cipaglucoSIDase alfa should be recorded in the infusion diary. In case of any problems with the reconstitution and administration of cipaglucoSIDase alfa, the infusion healthcare professional/nurse should consult with the treating physician to determine appropriate action before restarting or continuing the infusion.

The healthcare professional/nurse should ensure they provide the patient with the cipaglucoSIDase alfa patient information leaflet at the time of infusion. Patients should be advised to consult the information leaflet concerning potential adverse events and inform their home infusion healthcare professional/nurse, or treating physician directly, if they have any concerns about adverse events or receiving the treatment at home.

5

PREPARATION AND ADMINISTRATION OF CIPAGLUCOSIDASE ALFA

Instructions for use relating to the reconstitution, dilution and administration can be found in the Summary of Product Characteristics (SmPC).

The SmPC is available online at

<https://www.ema.europa.eu/en/medicines/human/EPAR/Pombiliti>

5.1

Prescription

The miglustat dose, cipaglicosidase alfa dose, required reconstituted volume, infusion rate, premedication, emergency medication, as well as any changes is determined by the treating physician and documented in the infusion diary. Any changes to this prescription must be recorded in the infusion diary.

Because cipaglicosidase alfa is administered in a weight-adjusted dose, it is important to check the patient's weight regularly to ensure correct dosing. The treating physician determines how to proceed in the case of a change in body weight. The infusion healthcare professional/ nurse must not change the dose of cipaglicosidase alpha or the infusion protocol prescribed by the treating physician, unless necessary due to safety considerations. To avoid medication errors, the correct determination of the required infusion volume is of great importance and should be determined in advance by the treating physician.

5.2

Supplies

TREATMENT DAY CHECKLIST



PRODUCTS

- ☐ **Cipaglicosidase alfa 105 mg vials** (20 mg/ kg body weight administered every other week)
- ☐ **Miglustat capsules** (for patients ≥ 50 kg, 4 capsules [260 mg total]; for ≥ 40 kg to < 50 kg, 3 capsules [195 mg total])
- ☐ Premedications as prescribed



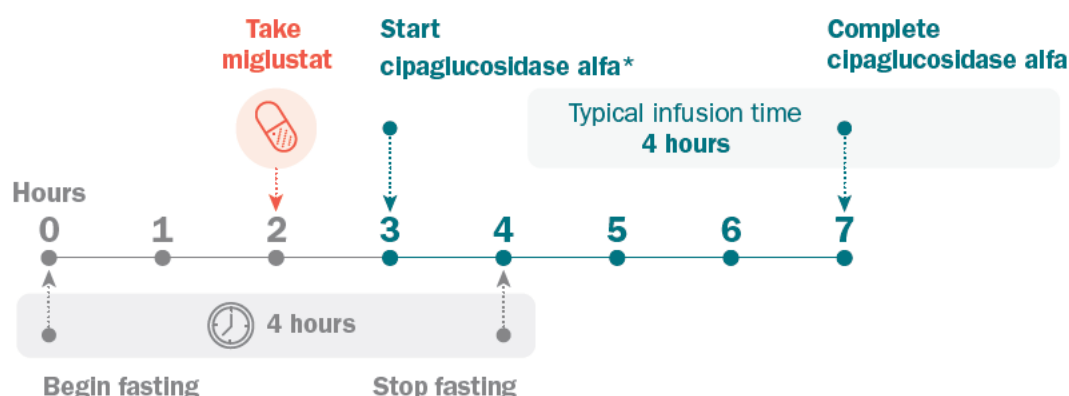
INFUSION SUPPLIES & EQUIPMENT

- ☐ **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

5.3

Timeline to administration including administration of miglustat

Cipaglucosidase alfa is only to be administered in conjunction with miglustat.

What the infusion day should look like:

* The cipaglucosidase alfa infusion should start 1 hour after taking miglustat capsules. In the event of infusion delay, the start of infusion should not exceed 3 hours from taking miglustat.



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

**DOUBLE-CHECK THE FOLLOWING**

- The patient has been fasting for 2 hours before and 2 hours after taking miglustat
- The patient has taken the miglustat capsules approximately 1 hour before the infusion is due to begin and it has not been more than 3 hours since they've taken miglustat prior to infusion initiation

5.3

(continued)

**TAKING MIGLUSTAT**

- Capsules should be taken on an empty stomach
- The patient must **fast 2 hours before and 2 hours after taking miglustat**
- Capsules should be taken approximately **1 hour before the start of cipaglucoSIDase alfa infusion**
 - In the event of infusion delay, the start of infusion should not exceed 3 hours from the oral administration of miglustat
- 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 miglustat

5.4

Dosing for both cipaglucoisidase alfa + miglustat is based on body weight

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

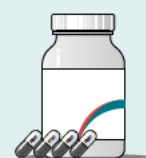
The treating physician must determine how to deal with the dosage in the event of a change in weight. To avoid medication errors, the correct determination of the required infusion volume is of great importance and should be determined in advance by the treating physician. The infusion healthcare professional / nurse should consult the treating physician to confirm the correct dose before initiating infusion.

Calculating the dose

Cipaglucoisidase alfa is administered to the patient by intravenous infusion every other week in conjunction with the oral medication miglustat.



Miglustat capsules of 65 mg are administered every other week in conjunction with the intravenous infusion of cipaglucoisidase alfa.



Weight-based dosing

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

Weight-based dosing

Recommended dosage:
 • ≥ 40 kg but < 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
Vials to reconstitute	Patient's dose (in mg) divided by 105 (mg/vial)	1300/105 mg per vial = 12.38 vials
Vials to prepare	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

5.5

Preparing for reconstitution

Before cipaglucosidase alfa 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 half an hour to reach room temperature of 20°C to 25°C.



ITEMS NEEDED FOR RECONSTITUTION AND DILUTION



Cipaglucosidase alfa
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 that has a
diameter of 18 gauge
or less

INSPECT EACH DRY POWDER VIAL CAREFULLY



Do not use if the lyophilised powder is discoloured, or if the closure is damaged or the button of overseal is removed.

5.6

Reconstituting the lyophilised cake/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 the inside of the vial rather than directly 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 discolouration. The reconstituted volume appears as a colourless-to-pale-yellow solution, clear to opalescent, and appears almost free of particles but may contain white-to-translucent particles.

- If upon immediate inspection foreign matter is observed, or if the solution is discoloured, do not use.
- 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.

5.7

Diluting the solution

- 1** Before dilution, 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 cipaglucosidase alfa solution for IV infusion.



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 cipaglucosidase alfa.



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.



Slowly inject reconstituted cipaglucosidase alfa 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.



- 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.

- 6** Repeat steps on remaining infusion bag(s) to achieve the total volume (mL) of reconstituted cipaglucosidase alfa required for the patient's dose.

Cipaglucosidase alfa should be administered as stated in the Summary of Product Characteristics



- 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 it is not possible to start the infusion following dilution, the diluted solution is stable for up to 24 hours refrigerated at 2°C to 8°C followed by 6 hours at room temperature (20°C to 25°C) to allow for infusion. Storage at room temperature is not recommended, refer to the SmPC. Do not freeze or shake.

5.8

Administering the Infusion



Now you're ready to begin the Infusion
The infusion will take approximately 4 hours.



Prior to infusion, inspect the infusion bag for foaming and if foaming is present, let foaming dissipate. Avoid shaking and handle infusion bag gently to prevent foaming.

1. The infusion solution should be administered at room temperature.
2. Total volume of infusion is determined by body weight and typically administered over approximately 4 hours.
3. Infusion of cipaglucosidase alfa should start approximately **1 hour after oral administration of miglustat**.
4. In the event of infusion delay, the **start of infusion should not exceed 3 hours from the oral administration of miglustat**.
5. The initial infusion rate should be no more than 1 mg per kg per hour for 30 minutes.
6. The infusion rate may be increased by 2 mg per kg per hour every 30 minutes, after patient tolerance to the infusion rate is established, until a maximum rate of 7 mg per kg per hour is reached.
7. Vital signs should be obtained at the end of each step.

Recommended Infusion Duration and Assessments

Step (Infusion Rate)	Cumulative Time (minutes)	Clinical assessment
Step 1 (1 mg/kg/hr)	0 – 30	Monitor for symptoms/ signs of IARs/ hypersensitivity (e.g. resp rate, HR, rash etc) Increase by 2 mg/kg/hr if tolerated
Step 2 (3/mg/kg/hr)	30 – 60	Monitor for symptoms/ signs of IARs/ hypersensitivity (e.g. resp rate, HR, rash etc) Increase by 2 mg/kg/hr if tolerated
Step 3 (5/mg/kg/hr)	60 – 90	Monitor for symptoms/ signs of IARs/ hypersensitivity (e.g. resp rate, HR, rash etc) Increase by 2 mg/kg/hr if tolerated
Step 4 (7/mg/kg/hr)	90 – 120	Monitor for symptoms/ signs of IARs/ hypersensitivity (e.g. resp rate, HR, rash etc)

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 (Section 6).

6

RECOGNISING AND MANAGING MEDICATION ERRORS, INFUSION-RELATED REACTIONS AND HYPERSENSITIVITY REACTIONS

Medication Errors

A medication error is an unintended failure in the drug treatment process that leads to, or has the potential to lead to, harm to the patient. The most common include mistakes in the prescribing, dispensing, storing, preparation and administration of a medicine.*

Infusion-related reactions and hypersensitivity reactions

Infusion-related reactions (IARs) and hypersensitivity reactions have been known to occur during or up to a few hours after the infusion of cipaglucoSIDase alfa and other enzyme replacement therapies (ERTs). Most IARs reported with cipaglucoSIDase alfa were mild or moderate in severity and transient in nature, and included abdominal distension, chills, dizziness, dysgeusia, dyspnoea, flushing, pruritus, pyrexia, and rash. Severe IARs, such as anaphylaxis, have also been reported in patients treated with cipaglucoSIDase alfa. Symptoms of severe IARs may include angioedema, bronchospasm, chest pain, chills, dyspnoea, generalised hives, hypotension, laryngeal oedema, syncope and tachycardia.

Management of infusion-related reactions

Premedication with oral antihistamine, antipyretics, and/or corticosteroids may be administered to assist with signs and symptoms related to IARs experienced with prior ERT treatment.

The recommendations on how to proceed in the event of an IAR are presented below.

The treating physician makes a final decision regarding treatment in the emergency plan.

Ensure immediate readiness for resuscitation before the start of the infusion.

Record on infusion diary all details regarding times, adverse reactions, medications and infusion rates.

*EMA, 2015. Good practice guide on recording, coding, reporting and assessment of medication errors (EMA/762563/2014).



Mild to moderate and transient IARs may be adequately managed by slowing the infusion rate or interrupting the infusion; medical treatment or discontinuation of cipaglicosidase alfa may not be required.

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.

In the event of anaphylaxis or severe allergic reactions, the infusion health professional/nurse must immediately pause the infusion and initiate appropriate medical treatment. 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 cipaglicosidase alfa following anaphylaxis or severe infusion reaction should be carefully considered, and if the patient continues therapy, their next infusion must occur in a clinical setting, equipped to deal with such medical emergencies.

Please also refer to the current Summary of Product Characteristics (SmPC) section 4.4 for complete information on the safety of cipaglicosidase alfa, available at <https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>

7

REPORTING ADVERSE REACTIONS

- ▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information.

Post-authorisation reporting of suspected adverse reactions is important. It allows continuous monitoring of the benefit/risk balance of the medicinal product.



The treating physician or home infusion healthcare professional/nurse should report any suspected adverse reactions to Amicus Therapeutics:

Tel: 1800 936 230.

Alternatively, send via e-mail to drugsafety@amicusrx.com.

Healthcare professionals are asked to report any suspected adverse reactions using the HPRA Website: www.hpra.ie.

If the patient or caregiver becomes aware that a mistake was made in the preparation and/or administration of the drug, the patient or infusion healthcare professional/nurse should inform the treating physician to determine appropriate action.



The treating physician or infusion healthcare professional/nurse should report any medication error as a spontaneous report to Amicus Therapeutics:

Tel: 1800 936 230.

Alternatively, send via e-mail to drugsafety@amicusrx.com.

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FURTHER INFORMATION



Please refer to the prescribing information for cipaglucosidase alfa and for miglustat for complete information on the safe and approved use of cipaglucosidase alfa in combination with miglustat.

The SmPC for cipaglucosidase alfa and miglustat are available on the EMA Website:

- cipaglucosidase alfa: <https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>
- miglustat: <https://www.ema.europa.eu/en/medicines/human/EPAR/opfolda>

Alternatively, the prescribing information can be requested at Amicus Therapeutics on

Tel: **1800 936 230**

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