

CIPAGLUCOSIDASE ALFA HOME INFUSION THERAPY

Patient/caregiver's guide including an infusion diary

VERSION 1.0

Patient/caregiver's guide including an infusion diary v1.0 has been approved by the
HPRA March 2026

Some people may have side-effects when taking this medicine. If you have any unwanted side-effects you should talk to your doctor, pharmacist or other healthcare professional. This includes any possible side-effects not listed in this leaflet. Also, you can help make sure medicines remain as safe as possible by reporting unwanted side-effects using the HPRA Website: www.hpra.ie. Alternatively, you can also report side-effects to Amicus Medical Information on: Tel: 1800 936 230; or email: drugsafety@amicusrx.com.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See www.hpra.ie for how to report side effects.

IMPORTANT

This patient guide contains important safety information you need to be aware of when receiving treatment with cipagluco­sidase alfa /miglustat. Read all of this information carefully before you start home infusion.

Keep this information in an easily accessible place; you may need to read it again.

- If you have further questions, ask your treating physician.
- This medicine has been prescribed for you. Do not pass it on to others even if their symptoms are the same as yours as it may harm them.
- If you experience any side effects, you and/or your caregiver must notify your treating physician or infusion nurse.
- For further information please refer to the package leaflet provided with your medicine and available online at:
<https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>

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Infusion diary for cipaglucosidase alfa home infusion

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YOUR DISEASE, TREATMENT AND HOME INFUSION

Together with your treating physician, you have decided to start home infusion therapy with cipaglucoaldase alfa. The objective of this document is to provide you with guidance on how to receive cipaglucoaldase alfa at home. The processes presented in this document serve as overall guidance but are subject to local medical practice and national rules and regulations. Your treating physician will provide you with the details that are applicable to your situation.

1.1

Pompe Disease and Treatment

Pompe disease is a very rare genetic disorder. It affects how your body uses glycogen, a type of carbohydrate stored in cells for energy. Normally, there's an enzyme called alpha-glucosidase that helps break down glycogen into glucose, which our body can use for energy. But in Pompe disease, there isn't enough of this enzyme, so glycogen builds up in different parts of your body, especially in the muscles. This causes the cells not to work properly and makes the muscles weak, which can lead to serious problems if not treated. The Pompe disease enzyme, α -glucosidase (also known as GAA), is one of those that helps to break down a substance called glycogen.

Pompe disease is usually treated by giving an infusion of enzyme replacement therapy (ERT), so that the glycogen is not allowed to build-up in your cells. ERT works by replacing the non-functioning or missing GAA with functioning GAA. Cipaglucoaldase alfa is a new ERT – this replaces the enzyme that is insufficient in Pompe disease. It is delivered by an infusion and has been specially designed to target the cells in your muscles. Miglustat is a capsule that you swallow before the administration of intravenous cipaglucoaldase alfa. This helps to ensure that the ERT gets to where it needs to be, in the muscles, so it can help clear the build-up of glycogen.

Refer to the cipaglucoaldase alfa and miglustat Package Leaflet for additional information.

The Package Leaflet can be found inside the product packaging and can also be accessed online at <https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti>

1.2**Home Infusion**

It may be possible for people suffering from Pompe disease and treated with cipaglucosidase alfa to receive their infusions at home.

- **The decision to receive home treatment should be made by you and your treating physician after initial infusions at the hospital or clinic to make sure you have no problems with the infusion. Typically, your physician will want to ensure that you are tolerating your infusions well in a hospital or clinic setting for a few months before deciding you are eligible for home infusion.**
- **The home infusion will take place under the direction of your treating physician and will be delivered by an appropriately trained infusion healthcare professional/nurse who will monitor you during and after each infusion.**

1.3**Information on signs and symptoms related to infusion related reactions**

Like all medicines, this medicine can cause side effects, although not everybody gets them. In clinical studies side effects were mainly seen while patients were being given the medicine or shortly after ("infusion related reactions"). Most of the infusion-related reactions were mild or moderate. Some of these infusion-related side effects may become serious and life-threatening.

→ **Symptoms of serious or life-threatening infusion-related reactions include:**

difficulty breathing, bloating, fever, chills, dizziness, skin redness, itchy skin and rash

→ **Symptoms of serious or life-threatening allergic reactions include:**

rash, puffy eyes, prolonged difficulty breathing, cough, swelling of the lips, tongue or throat, itchy skin and hives

1.3

(continued)

Your doctor may decide to lower the infusion rate or give you additional medicines to prevent such reactions from occurring again.



For the full list of all side effects reported with ctpaglucoaldase alfa, see the Package Leaflet.

1.4

Recommended actions for the management of the ADRs when symptoms occur

- In the event that you do not feel well due to the medication during the home infusion or shortly after the infusion, you must ask your home infusion provider to immediately stop the medication. The treating physician or the emergency services (see instructions in the infusion diary) must be contacted immediately. Subsequent infusions may need to occur in a hospital or clinic setting until your prescribing physician is satisfied that you are tolerating the infusions well.
- If you feel unwell for any other reason (e.g. have a cold or flu-like illness) you should inform your physician or home infusion nurse. Your infusion may need to be rescheduled if you are otherwise unwell.
- Any symptoms or side effects should also be recorded in the infusion diary.

1.5

Reporting Side-effects

Some people may have side-effects when taking these medicines. If you have any unwanted side-effects you should talk to your doctor, infusion healthcare professional/nurse or other healthcare professional. This includes any possible side-effects not listed in the Package Leaflet



Also, you can help make sure medicines remain as safe as possible by reporting any unwanted side-effects. You can report side effects using the HPRA website: www.hpra.ie. Alternatively, you can also report side-effects to Amicus Medical Information on: Tel: 1800 936 230; or email: drugsafety@amicusrx.com

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ORGANISATION OF TREATMENT AT HOME

2.1

Pre-treatment and Emergency Treatment

- If necessary, your treating physician will prescribe pre-treatment medication. Your treating physician will include the information on this medication in the Infusion Diary
- Your treating physician will prescribe medication to respond to an emergency situation, if necessary. Your treating physician will include the information on this medication in the Infusion Diary. This emergency medication should be available during the infusions at home.

2.2

The Infusion Diary

- You have been provided the Patient/caregiver's guide including an Infusion Diary.
- The Infusion Diary will be used to record the infusions and document any infusion associated side effects, including allergic-type hypersensitivity reactions before, during or after the infusion.
- This will ensure that relevant information is readily available to healthcare professionals administering Pombiliti.

INFUSION DIARY FOR cipaglucosidase alfa home infusion

Some people may have side-effects when taking this medicine. If you have any unwanted side-effects you should talk to your doctor, pharmacist or other healthcare professional. This includes any possible side-effects not listed in this leaflet. Also, you can help make sure medicines remain as safe as possible by reporting unwanted side-effects using the HPRA Website: www.hpra.ie. Alternatively, you can also report side-effects to Amicus Medical Information on: Tel: 1800 936 230; or email: drugsafety@amicusrx.com.

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PATIENT DETAILS

Name	
Address	
Eircode	
Contact Details	

TREATING PHYSICIAN DETAILS

Name	
Hospital/Clinic	
Contact Details	

INFUSION HEALTHCARE PROFESSIONAL/ NURSE

Name	
Hospital/Clinic	
Contact Details	

PATIENT NEXT OF KIN

Name	
Contact Details	

ADMINISTRATION DETAILS (TO BE COMPLETED BY TREATING PHYSICIAN)

Date of first administration	
First infusion at home	

CIPAGLUCOSIDASE ALFA/MIGLUSTAT DOSING REGIMEN

Miglustat dose	
Cipaglucosidase alfa dose	
Frequency	
Rate of infusion	
Required reconstituted volume (mL)	
Total volume in infusion bag (mL)	
Pre-treatment medication (if applicable)	
Reasons for cipaglucosidase alfa infusion at home	
Additional notes	

NECESSARY ACTIONS IN THE EVENT OF A SEVERE INFUSION-ASSOCIATED REACTION OR HYPERSENSITIVITY REACTION

(TO BE COMPLETED BY TREATING PHYSICIAN)

1

STOP the infusion

2

Call the emergency services

3

Call your treating physician

4

Emergency medication

Medication, including dose

5

Patient's next of kin to be contacted

COMPLETE THIS FORM FOR EVERY INFUSION SESSION

The patient and/or caregiver(s) have been informed about the associated risks of home infusion of cipaglucoisidase alfa, and proper education on the use of emergency medications has been provided. In the event of any infusion-associated reaction, the infusion must be immediately discontinued. Necessary actions in the event of a severe infusion-associated reaction, including emergency contact details, are described in this Infusion Diary. Keep this information readily available during the infusion procedure.

Date of first administration	
First infusion at home	

INFUSION SESSION	
Date of infusion	
Infusion healthcare professional/nurse	
Caregiver, if present	

DOSE	
Miglustat dose	
Time of miglustat dose	
Cipaglucoisidase alfa dose	
Patient weight (kg)	
Cipaglucoisidase alfa batch/lot number	
Time of cipaglucoisidase alfa infusion start	
Required reconstituted volume (mL)	
Number of vials used	
Duration of infusion	
Rate(s) of infusion	
Problems/Remarks related to the infusion, if any (including infusion- associated reaction(s), action taken, and outcome):	
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