Manual for patients and caregivers with Mucopolysaccharidosis Type I (MPS I) Disease who receive home infusion of Aldurazyme® (laronidase)

This material fulfils the conditions of the marketing authorisation and has been approved by the Medicines and Healthcare products Regulatory Agency (MHRA).

VV-PV-0619971 (v1.0) | DATE OF PREPARATION: DECEMBER 2025
DATE OF MHRA APPROVAL: DECEMBER 2025

sanofi

About this document

Read all of this information carefully.

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

- If you have further questions, ask your prescribing physician and the healthcare professional (HCP) administering the infusion.
- This medicine has been prescribed for you or your dependent. Do not pass it on to others even if their symptoms are the same as the patient's as it may harm them.
- If you experience any side effects, you and/or your caregiver must notify your prescribing physician or infusion HCP.

Table of Contents

ABBREVIATIONS

CNS : Central Nervous System

GAGs : Glycosaminoglycans

HCP : Health Care Professional (doctor, nurse, others)

IAR : Infusion Associated Reaction

MPS I : Mucopolysaccharidosis type I

PIL : Patient Information Leaflet

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

1. MPS I DISEASE AND TREATMENT

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

MPS I is a rare, inheritable, genetic condition that may present itself in both children and adults. **MPS disease** is a rare disease in which first symptoms can become evident at any age from birth to early adulthood, but usually doctors are able to identify symptoms started early in life, but as these symptoms are quite common in children, they get overlooked.

Patients with MPS I disease have low or absent levels of an enzyme called 'alpha-L-iduronidase'. This enzyme is normally responsible for the breakdown of complex sugary compounds named "glycosaminoglycans" or "GAGs", and as a result, abnormal deposits of GAGs build up in organs, affecting most systems of the body, causing damage to the tissues and hampering its functionality.

Laronidase is an artificially produced enzyme which is intended to replace the natural enzyme alpha-L-iduronidase that has insufficient or absent function in patients with MPS I disease. Laronidase is used for the long-term treatment of the manifestations caused by the MPS I disease in those individuals with a confirmed diagnosis. It is important to note that laronidase can't address the neurological manifestations of the disease, because it can't cross the natural barrier that protects the central nervous system (CNS).

Refer to the Patient Information Leaflet (PIL) for additional information.

2. HOME INFUSION

Currently, in some countries, people suffering from MPS I disease and treated with laronidase, receive their infusions at their home. Your doctor will assess if home infusion is appropriate for you or the patient under your care. **The decision to receive home treatment should be made by the prescribing physician and you/the caregiver, with a period of initial infusions performed at the hospital to make sure you or the patient under your care have no problems during the infusions.**

Home infusion of laronidase allows you or the patient under your care to receive treatment at your/their own living environment which increases comfort and flexibility of infusion timing. It also prevents the patients from travelling to the hospital every week. Patients that receive treatment at home may follow their normal schooling program and/or organize social and professional activities more easily. Home infusion may also simplify arranging treatment around family and friends.

Home infusion is the responsibility of the prescribing physician. The physician has given you this patient/caregiver guide because of their belief that you or the patient under your care, can have laronidase administered at home and this is also the preferred option. **It is the responsibility of the prescribing physician to ensure a safe administration to the patient.** This should be checked and documented by the prescribing physician. The patient and their family/caregiver should consent to transitioning to home infusion, and the home facility must be suitable to perform the infusions.

An appropriately trained infusion HCP will perform the infusion at the patient's home.

2.1 Organisation

Patient

- You and/or your caregiver must agree to receive the treatment at home.
- The home environment should be conducive to the provision of the home infusion therapy including a clean environment with electricity, water, telephone access, refrigeration, and physical space to support storage of laronidase and other infusion supplies.
- You must be physically and mentally able to undergo the infusions at home. The prescribing physician is responsible for the indication to receive laronidase infusions at home.
- You have accessible blood veins that allow an infusion needle to be inserted.
- You and/or your caregiver have been informed by the prescribing physician about the treatment to be provided at home, the associated risks, the possible complications, and the provision of medical assistance at home.
- You and/or your caregiver have knowledge of MPS I disease and are able to recognise side effects and understand the procedures to be followed should they occur.

Infusion HCP

- The infusion HCP is qualified to give intravenous (IV) infusions.
- The infusion HCP has been trained in administering laronidase and is aware of the possible side effects and the actions to be taken should they occur.
- The infusion HCP will establish with the patient and/or caregiver the level of support necessary.
- The infusion HCP will have a coordinating task together with the prescribing physician and you and/or your caregiver in organising the treatment at home.
- The infusion HCP will strictly follow the prescribed dose and rate of administration of laronidase as stated in the Infusion Diary.
- The infusion HCP and/or patient/caregiver will record each administration of laronidase in the Infusion Diary.

- In the event of a side effect occurring during or shortly after the infusion (i.e., infusion associated reaction), the infusion HCP/patient/caregiver should discontinue the infusion and phone the prescribing physician and/or the emergency services number provided in the Infusion Diary.

Prescribing physician

- The prescribing physician is responsible for the initiation of all necessary administrative actions, allowing other stakeholders (pharmacy, nurse, patient, caregiver) to proceed.
- The prescribing physician is responsible for the dose and the infusion rate, to be described in the Infusion Diary. Any changes must be clearly communicated to the patient and/or caregiver, and infusion HCP and described in the Infusion Diary.

Caregiver

- It is preferable for a caregiver to be present during home infusion.

The Infusion Diary (Appendix 4.1)

- The Infusion Diary serves as a means of communication for everyone involved in administering laronidase at home.
- The Infusion Diary should be kept at your home and will be kept up to date by you, your caregiver or the infusion HCP.
- In the Infusion Diary, the prescribing physician clearly states the dose and the infusion rate, as well as any changes.
- The infusion HCP records the findings and actions from the initial interview and you, your caregiver or the infusion HCP notes all relevant information from subsequent visits in the Infusion Diary.
- Infusion HCP will strictly follow the prescribed dose and rate of infusion of laronidase as stated in the Infusion Diary.
- You and/or your caregiver and/or infusion HCP will record each administration of laronidase in the Infusion Diary.
- You and/or your caregiver must take the Infusion Diary along to the hospital at each appointment for a check-up and bring it home afterwards.
- In the Infusion Diary, the patient/caregiver/infusion HCP clearly describes what actions have been taken for the infusion side effect based on the advice of the prescribing physician or the infusion HCP.

Pharmacy and infusion equipment

- Treatment and all necessary equipment will be provided according to the local requirements.

3. SIDE EFFECTS MANAGEMENT

Like all medicines, laronidase treatment may cause side effects, although not everybody experiences them.

Side effects were mainly seen while patients were receiving the infusion of laronidase or shortly after ("infusion associated reactions" (IARs)).

Some of these IARs were serious or life-threatening. Life threatening reactions, including very severe generalized allergic reactions and anaphylactic shock, have been reported in some patients. Symptoms may include inability of lungs to work properly (respiratory failure/distress), high-pitched breathing sound (stridor) and other disorders due to obstruction of airways, rapid breathing, excessive contraction of the airway muscles causing breathing difficulty (bronchospasm), lack of oxygen in body tissues (hypoxia), low blood pressure, slow heart rate, itchy rash (urticaria), swelling of the throat or face. These reactions may be particularly severe if you have a pre-existing MPS I-related upper airway obstruction.

Some patients have experienced infusion related side effects in the form of flu-like symptoms, which lasted for a few days after completion of the infusion.

Beware that some patients have experienced side effects several hours after the infusion ended and that it can be experienced at any time, even months after being stable on the infusion.

For a full list of possible side effects, read the PIL that came with this medicine.

Should you or the patient under your care experience any reaction please contact your/their prescribing physician immediately.

Reporting of side effects:

Please report side effects to the MHRA through the Yellow Card scheme.
You can report via:

- the Yellow Card website www.mhra.gov.uk/yellowcard
- the free Yellow Card app available from the Apple App Store or Google Play Store

Alternatively you can report suspected side effects to the Yellow Card scheme by calling 0800 731 6789 for free, Monday to Friday between 9am and 5pm. You can leave a message outside of these hours.

When reporting please provide as much information as possible. By reporting side effects, you can help provide more information on the safety of this medicine.

Side effects can also be reported to Sanofi: Tel: 0800 090 2314;
email: uk-drugsafety@sanofi.com

The prescribing physician will decide how to continue with the treatment, or if you or the patient under your care needs to receive pre-treatment medication to prevent some of these adverse reactions (e.g. antihistamines, corticosteroids and/or antipyretics). In some instances, the prescribing physician may decide to continue treatment at the hospital until your or the patient under your care's safety has been ensured, or even revert infusions in the hospital permanently.

It is possible that the prescribing physician has decided to give you or the patient under your care other medicines to prevent mild and moderate reactions.

If you or the patient under your care have a severe side effect during an infusion, the infusion HCP will stop the infusion and follow the guidance provided by the prescribing physician.

In case of a mild or moderate side effect, the infusion HCP will stop temporarily the infusion and restart at a lower infusion rate depending on the persistence or not of the symptoms. The infusion HCP may consider administering additional medication. If the symptoms don't disappear, the infusion HCP might decide to fully stop the infusion for that day.

For the full list of all side effects reported with laronidase, refer to the PIL.

In the event that you or the patient under your care does not feel well due to the medication during the home infusion, the medication will be immediately stopped by the infusion HCP. The prescribing physician, their medical designate, and/or the country-specific national emergency number might be contacted immediately depending on the severity of the side effect. Subsequent infusions may need to occur in a clinical setting.

The prescribing physician and/or emergency services must also be phoned if any IAR, particularly hypersensitivity reactions, occurs shortly after completion of the infusion. Any IAR must be recorded in "The Infusion Diary", included at the end of this guide.

4. THE USE OF "THE INFUSION DIARY"

As an **appendix** to this patient guide is included ***"The Infusion Diary"***.

The Infusion Diary is where you may record all your or the patient under your care's infusions and any side effects, during or after the infusion.

Appendix 4.1: The Infusion Diary

Infusion Diary for recording the infusions with laronidase General data to be completed by the prescribing physician. Emergency Number:	
Patient:	Name:
	Address:
	Post code/City:
	Phone number:
Patient caregiver:	Name:
	Address:
	Post code/City:
	Phone number:
Infusion HCP (the HCP administering laronidase)	Name:
	Institution:
	Post code/City:
	Phone number:
Prescribing physician (the HCP prescribing laronidase)	Name:
	Institution:
	Post code/City:
	Phone number:
	Emergency number:

Administration details (to be completed by prescribing physician)

Laronidase administered since (dd-mmm-yyyy)	
First infusion at home (dd-mmm-yyyy)	
Reasons for laronidase infusion at home	
Please indicate support to be provided by infusion HCP	

Emergency treatment details (to be completed by prescribing physician)

Necessary actions in the event of a serious infusion associated reaction: 1. Stop the infusion. 2. Call the emergency services number. 3. Call the prescribing physician.
Further notes:

Infusion data (to be completed by infusion HCP and/or patient and/or caregiver)

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions (infusion HCP)	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	

Date of infusion: (dd-mmm-yyyy)	
Name of the health care professional administering the infusions	
Laronidase dosing regimen (dose, frequency, and rate of infusion) as per most recent prescription	
Patient's general health condition: specific problems/ remarks	
Batch number/ Number of vials used	
Duration of administration	
Any reactions to the infusion?	
During the infusion?	__Yes __No
After the infusion?	__Yes __No
Comments:	