Aldurazyme® (laronidase)
Home Infusion Therapy

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

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This document is approved by The Executive Directorate of Pharmacovigilance, at SFDA.

The patient/caregiver guide contains the following elements:

- Information on the risk of Infusion Administration Reactions notably hypersensitivity and anaphylactic reactions, including their signs and symptoms and the recommended actions when symptoms occur.
- An Appendix with the Patient/Caregiver's diary to follow up infusions

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 treating physician and the healthcare professional 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 treating physician or infusion HCP.

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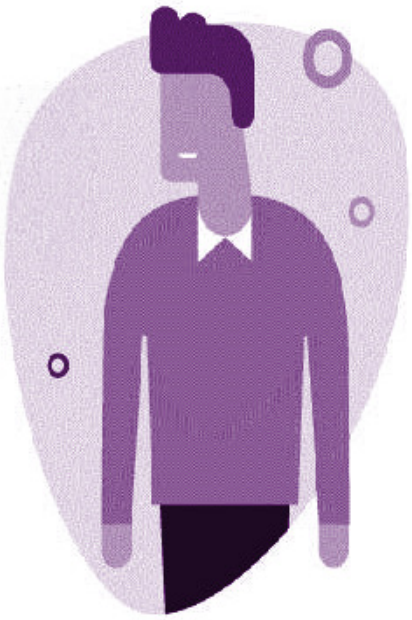
ABBREVIATIONS

MPS I : Mucopolysaccharidosis type I
GAGs : Glycosaminoglycans
CNS : Central Nervous System
HCP : Health Care Professional (doctor, nurse, others)

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

01 MPS I DISEASE AND TREATMENT



Together with your treating physician, you have decided to start home infusion therapy with Aldurazyme®. The objective of this document is to provide you with guidance on how to receive Aldurazyme® 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.

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.

Aldurazyme® is an artificially produced enzyme called laronidase which is intended to replace the natural enzyme alpha-L-iduronidase that has insufficient or absent function in patients with MPS I disease. Aldurazyme 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 Aldurazyme 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 Package Leaflet and the Summary of Product Characteristics (SmPC) of Aldurazyme for additional information.

02
Home Infusion

Currently, in some countries, people suffering from MPS I disease and treated with Aldurazyme, receive their infusions at their home. **The decision to receive home treatment should be made by your treating physician and you/the caregiver, with a period of initial infusions performed at the hospital to make sure you have no problems during the infusions.**

Home infusion of Aldurazyme allows you to receive your treatment at your 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 also simplifies arranging treatment around family and friends.

Home infusion is the responsibility of the treating physician. Your physician has given you this patient/caregiver guide because of his/hers belief that you or the patient under your care, can have Aldurazyme administered at home and you also prefer this option. **It is the responsibility of the treating physician to ensure a safe administration to the patient.** This should be checked and documented by the treating physician. The patient and their family 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 your home.

03 Adverse events management

Like all medicines, Aldurazyme treatment may cause adverse reactions, although not everybody experiences them.

Side effects were mainly seen while patients were receiving the infusion of Aldurazyme or shortly after ("infusion related reactions").

Some of these infusion related reactions were serious or life-threatening. Life threatening reactions, including very severe generalized allergic reactions and anaphylactic shock, have been reported in some patients. Symptoms of such reactions include low blood pressure, very fast heart rate, difficulty in breathing, vomiting, facial, lip or tongue swelling, hives or rash.

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 adverse reactions several hours after the infusion ended.

Should you experience any reaction please contact your doctor immediately.

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

It is possible that your treating physician has decided to give you other medicines to prevent mild and moderate reactions.

If you have a severe adverse reaction during an infusion, your infusion HCP will stop

the infusion and follow the guidance provided by your treating physician.

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

Very common side effects:

headache, nausea, abdominal pain, rash, joint disease, joint pain, back pain, pain in arms or legs, flushing, fever, chills, increased heart rate, increased blood pressure, and reaction at the infusion site.

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

In the event that you do not feel well due to the medication during the home infusion, the medication will be immediately stopped by the infusion healthcare professional and contact the treating physician for medical assistance immediately depending on the severity of the adverse reaction. Subsequent infusions may need to occur in a clinical setting.

If an IAR occurs shortly after completion of the infusion. Any IAR must be recorded in the "Patient Diary", included at the end of this guide.

04

The use of "The Infusion Diary"

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

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

Should you experience any reaction, please tell your doctor immediately. You can also report directly to your local reporting system In case of any drug related adverse events, please contact:

The National Pharmacovigilance Centre (NPC- Saudi Food and Drug Authority (SFDA):
Call Center: 19999
E-mail: npc.drug@sfda.gov.sa
Website: https://ade.sfda.gov.sa/

For adverse event reporting, please contact:
Sanofi Pharmacovigilance Department
Telephone: +966 54 4284 797, Fax: +966-11-205-7662
Email: Ksa_pharmacovigilance@sanofi.com
For extra copies please contact (+966 501736796)

Annex

The Infusion Diary

The Infusion Diary

Infusion Diary for recording the infusions with Aldurazyme® (laronidase)
General data to complete by the treating physician.

Emergency Number:	
Patient:	Name:
	Address:
	City:
	Phone number:
Patient caregiver:	Name:
	Address:
	City:
	Phone number:
HCP administering Aldurazyme	Name:
	Institution:
	City:
	Phone number:
Treating physician (the HCP prescribing Aldurazyme)	Name:
	Institution:
	Phone number:
	City:
	Emergency Number:

The Infusion Diary

Date of infusion:	
Name of the health care professional administering the infusions	
Any reactions to the infusion?	
During the infusion?	☐ No ☐ Yes
After the infusion?	☐ No ☐ Yes
Comments:	

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Any reactions to the infusion?	
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