

Aldurazyme® (Iaronidase)

Home Infusion guide for patients and their caregivers -

Aldurazyme treatment of Mucopolysaccharidosis Type I (MPS I) Disease

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The patient/caregiver guide contains the following elements:

- *Information about MPS I and home infusion.*
- *Information on the risk of Infusion Associated Reactions (IARs), notably hypersensitivity and anaphylactic reactions, including their signs and symptoms and the recommended actions if symptoms occur.*

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 Doctor and the Infusion Nurse giving you 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 Doctor or Infusion Nurse.

Contents

1	ABOUT THIS GUIDE.....	4
1.1	INTRODUCTION TO MPS I1.....	4
1.2	INTRODUCTION TO ALDURAZYME ¹	4
2	WHAT CAN I EXPECT FROM TREATMENT WITH ALDURAZYME AT HOME?	6
2.1	BEFORE YOU CAN START HOME INFUSIONS	7
3	HOW ARE ALDURAZYME SIDE EFFECTS MANAGED WITH HOME INFUSION?	8

ABBREVIATIONS

MPS I : Mucopolysaccharidosis type I

GAGs : Glycosaminoglycans

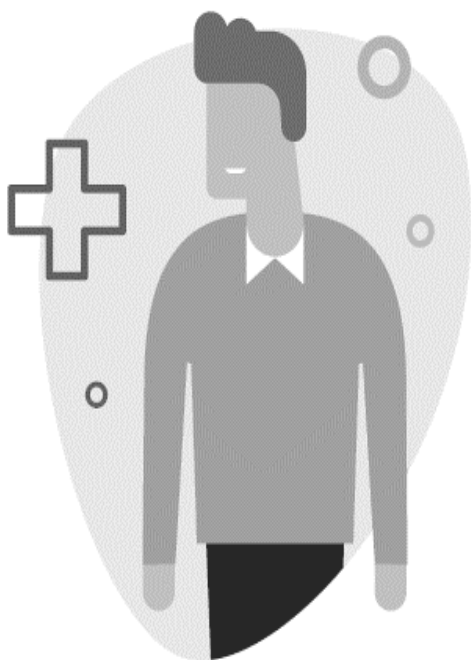
CNS : Central Nervous System

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

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

1 ABOUT THIS GUIDE

Together with your Doctor, you have decided to start home infusion therapy with Aldurazyme®. This guide provides you with information on how you will receive Aldurazyme® at home, but it does not replace the advice from your healthcare team. If you have any questions or concerns about Aldurazyme home infusions, please talk to your healthcare professional.



1.1 INTRODUCTION TO MPS I1

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

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

1.2 INTRODUCTION TO ALDURAZYME¹

Aldurazyme® is an enzyme replacement therapy that replaces the natural enzyme alpha-L-iduronidase, the enzyme that people living with MPS are lacking. By providing this enzyme as a medicine, the buildup of GAGs in the body can be reduced.¹ Aldurazyme doesn't treat the brain related effects because it can't cross the natural barrier that protects the central nervous system (CNS).

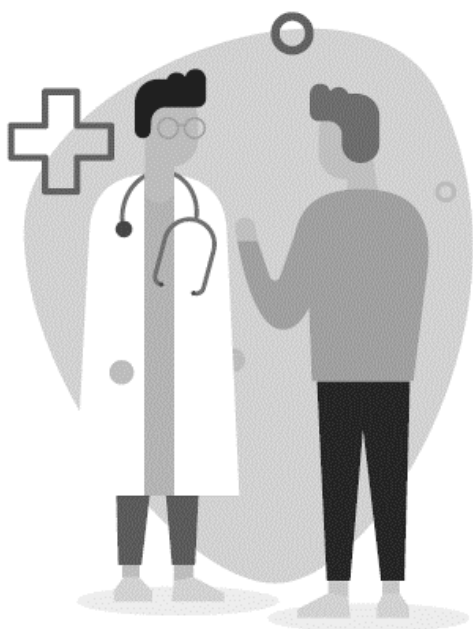
Talk to your healthcare professional about treatment with Aldurazyme and refer to the Aldurazyme Consumer Medicines Information (CMI)

for of Aldurazyme, available at www.tga.gov.au for additional information.

References: ¹Aldurazyme Approved Product Information

2 WHAT CAN I EXPECT FROM TREATMENT WITH ALDURAZYME AT HOME?

In Australia, people suffering from MPS I disease and treated with Aldurazyme can receive their infusions at their home. **The decision to receive the infusion at home should be made by your Doctor and you/the caregiver together, after a period of initial infusions performed at the hospital to make sure you that home infusions are suitable for you.**



Home infusion of Aldurazyme allows you to receive your treatment in the comfort of your own home, on a day suitable for you.

It saves time commuting and spent at the hospital, and allows you to schedule your infusions around your daily life commitments with school, social and professional activities more easily.

Home infusions will be given by home infusion nurses who are trained experts in enzyme

replacement therapy infusions.

Home infusions are the responsibility of your Doctor. Your Doctor will discuss home infusions with you and assess your suitability to receive infusions at home before referring you to the home nursing program.

It is the responsibility of your Doctor to ensure safe administration of Aldurazyme.

2.1 BEFORE YOU CAN START HOME INFUSIONS

- Your doctor will ensure you have been receiving infusions the hospital and tolerating the infusions well.
- Your doctor will confirm that you are medically stable and have not experienced any side effects to treatment that cannot be managed with pre-infusion medication.
- You can commit to regular infusions and appointments at your home.
- Your home is safe and suitable for the home infusion nurse to give you the infusions.

3 HOW ARE ALDURAZYME SIDE EFFECTS MANAGED WITH HOME INFUSION?

Like all medicines, Aldurazyme treatment may have unwanted side effects, although not everybody experiences them.

Side effects were mainly seen while patients were receiving their infusion of Aldurazyme or shortly afterwards, which is referred to as “infusion related reactions (IARs)”.

You may be at risk of developing these side effects. It is important you understand these risks and how to monitor for them.

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

Some patients have also experienced adverse reactions several hours after the infusion ended.



Some of these infusion associated reactions were serious or life-threatening. Life threatening reactions, including very severe generalised allergic reactions and anaphylactic shock, have been reported in some patients.

If you have any of these serious side effects during the infusion, tell your infusion nurse straight away.

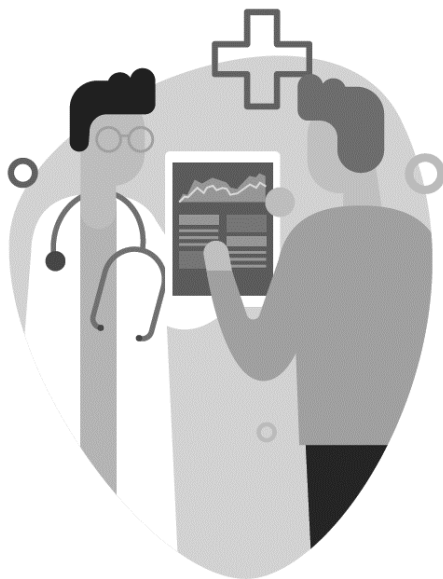
If it is after your infusion, call your doctor straight away, or call Triple Zero (000).

- Sudden signs of allergy such as rash, itching or hives on the skin
- Swelling of the face, lips, tongue or other parts of the body
- Shortness of breath, wheezing or trouble breathing
- Respiratory failure (inability of the lungs to work properly)

Your doctor will decide how to continue with the treatment, or if you need to receive pre-treatment medication to reduce the chance of some of these side effects (e.g. antihistamines, corticosteroids and/or antipyretics). In some instances, your doctor may decide to continue treatment at the hospital for a period of time, or even go back to infusions in the hospital permanently.

It is possible that your Doctor has decided to give you other medicines to prevent mild and moderate side effects.

If you have a severe side effect during an infusion, your Infusion Nurse will stop the infusion and follow the guidance provided by your Doctor.



In case of a mild or moderate side effect, your Infusion Nurse will temporarily stop the infusion and then restart it at a slower infusion rate, depending whether your side effects go away or not. Your Infusion Nurse may consider administering additional medication. If the symptoms don't disappear, your Infusion Nurse might decide to fully stop the infusion for that day.

For the full list of side effects reported with Aldurazyme, see the Aldurazyme Consumer Medicines Information, available at www.tga.gov.au.

If you feel unwell during the home infusion, your Infusion nurse will immediately stop your infusion.

Depending on the severity of the reaction, the Infusion nurse may immediately contact Triple Zero (000), or the treating doctor and/or their medical designate.

If an IAR occurs shortly after your infusion ends, the Infusion nurse may also immediately contact Triple Zero (000), the treating doctor and/or their medical designate.

Subsequent infusions may need to occur in the hospital.