



Guidelines for the treatment of mucopolysaccharidosis type I (MPS I) through the Life Saving Drugs Program

Life Saving Drugs Program

About this program

Through the Life Saving Drugs Program (LSDP), the Australian Government provides subsidised access for eligible patients to expensive life-saving medicines.

Purpose of this document

This document provides guidance for treating physicians with relevant specialist registration who wish to apply for their patients to receive access to subsidised treatment for MPS I through the LSDP.

It describes the criteria for general, initial and ongoing eligibility to access subsidised treatment and the administrative requirements associated with the initial application and annual reapplications.

Treatment of MPS I through the LSDP

Subsidised MPS I medicine treatment is available for eligible patients with a confirmed diagnosis of Hurler-Scheie Syndrome. Subsidised treatment through the LSDP is not available for patients with either Hurler Syndrome (MPS IH) or Scheie Syndrome (MPS IS).

Medicines currently available for the treatment of MPS I through the LSDP

There is one medicine currently subsidised through the LSDP for the treatment of MPS I.

The generic name for this medicine is laronidase. The trade name for this medicine is Aldurazyme®.

The Therapeutic Goods Administration (TGA) registration and Product Information for laronidase (Aldurazyme®) can be found on the [TGA's website](#).

Dosage

The maximum dosage of laronidase that is subsidised through the LSDP is 100 U/kg (0.58 mg/kg) weekly.

General eligibility requirements

LSDP funding conditions

A patient must continually meet the LSDP funding conditions in order to be eligible to receive access to Australian Government-subsidised treatment for MPS I through the LSDP.

The current LSDP funding conditions can be found on the [program's website](#).

For MPS I, a patient must:

- satisfy the initial and ongoing eligibility criteria as detailed in these guidelines
- participate in the evaluation of effectiveness of the medicine by periodic assessment, as directed by these guidelines, or have an acceptable reason not to participate
- not be suffering from any other medical condition, including complications or sequelae of MPS I, that might compromise the effectiveness of the treatment, and
- be an Australian citizen or permanent Australian resident who qualifies for Medicare.

In most cases, participation in a clinical trial will not affect a patient's eligibility to access LSDP medicines. However, treating physicians are required to advise the LSDP if their patient is participating in a clinical trial.

Exclusion criteria

The following patients are not eligible for subsidised treatment with laronidase for the treatment of MPS I through the LSDP:

- Patients with confirmed MPS IH or MPS IS
- Patients who have significant learning difficulties and/or neuropathic involvement with their disease indicating MPS IH
- Patients with another life threatening or severe disease where the long term prognosis is unlikely to be influenced by enzyme replacement therapy (ERT)
- Patients with another medical condition that might reasonably be expected to compromise a response to ERT.

Initial eligibility requirements

Diagnosis

The diagnosis of MPS I must be confirmed by the demonstration of a deficiency of alpha-L-iduronidase in white blood cells with the assay performed in a NATA-accredited laboratory; or, for siblings of a known patient, detection of 2 disease-causing mutations.

A deficiency of alpha-L-iduronidase in white blood cells should be confirmed by either an enzyme assay in cultured skin fibroblasts or by detection of 2 disease-causing mutations in the alpha-L-iduronidase gene.

The patient must present with at least one of the following complications of MPS I to be eligible for treatment with laronidase:

- **Sleep disordered breathing:** Patients with an apnoea/hypopnoea incidence of > 5 events/hour of total sleep time or > 2 severe episodes of desaturation (oxygen saturation < 80%) in an overnight sleep study.
- **Respiratory function tests:** Patients with FVC < 80% of predicted value for height.
- **Cardiac:** Myocardial dysfunction as indicated by a reduction in ejection fraction to < 56% (normal range 56-78%) or a reduction in fraction shortening to < 25% (normal range 25-46%).
- **Joint contractures:** Patients developing restricted range of movement of joints of > 10 degrees from normal in shoulders, neck, hips, knees, elbows or hands.
- Infants and children aged < 5 years: Applications may be submitted for infants and children not yet demonstrating symptoms consistent with other eligibility criteria where there has been a diagnosis of MPS I, for example by genotyping, with clear prediction of progress of the disease, or if severe disease can be predicted on the basis of a sibling's disease progression.

See the [initial application form](#).

Ongoing eligibility requirements

The treating physician must submit the separate [reapplication form](#) to the LSDP by 1 May every year if they wish their patient to continue to receive subsidised treatment through the LSDP.

If a reapplication is not submitted by 1 May each year without a clinical justification, the patient is at risk of having their treatment paused until the reapplication is received.

The reapplication form must demonstrate clinical improvement in the patient or stabilisation of the patient's condition, and evidence to support ongoing eligibility for the treatment of MPS I must be provided.

The treating physician must declare that the patient continues to meet the eligibility criteria to receive subsidised treatment through the LSDP in accordance with these guidelines.

The clinic letter and test results provided to support the reapplication must be no more than 12 months old at the time of each reapplication and should not have been used to support a previous application or reapplication.

Subsidised treatment may continue unless one or more of the following situations apply:

- failure to comply adequately with treatment or measures
- failure to provide data, copies of test results, and the [Excel spreadsheet for MPS I](#) evidencing the effectiveness of the therapy. Test results must not be more than 12 months old at the time of reapplication to the LSDP and should not have been used to support a previous application or reapplication
- therapy fails to relieve or stabilise the symptoms of disease that originally resulted in the patient being approved for subsidised treatment
- the patient has severe infusion-related adverse reactions which are not preventable by appropriate pre-medication and/or adjustment of infusion rates
- the patient develops significant learning difficulties and/or neuropathic involvement with their disease indicating MPS IH

- the patient develops another life threatening or severe disease where the long term prognosis is unlikely to be influenced by ERT
- the patient develops another medical condition that might reasonably be expected to compromise a response to ERT
- presentation of conditions listed in the exclusion criteria.

Testing is not funded or subsidised through the LSDP, however some tests may be subsidised through Medicare or available through the treating public hospital.

Cessation of treatment

The treating physician should notify the LSDP immediately in writing when a patient ceases treatment, including the reason(s) for treatment cessation.

Treatment breaks

Treatment breaks of up to 3 months can be taken without the requirement for submission of a new reapplication form to recommence treatment.

Patients who are applying to recommence treatment following a break of longer than 3 months should submit a new [reapplication form](#).