Australian Government
Department of Health and Aged Care

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

The 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 drugs.

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 II 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 II through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of MPS II.

Drugs currently available for the treatment of MPS II through the LSDP

There is one drug currently subsidised through the LSDP for the treatment of MPS II.

The generic name for this drug is idursulfase. The trade name for this drug is Elaprase®.

The Therapeutic Goods Administration (TGA) registration and Product Information for idursulfase (Elaprase®) can be found on the TGA's website.

Dosage

The maximum dosage of idursulfase that is subsidised through the LSDP is 0.5 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 II through the LSDP.

The current LSDP funding conditions can be found on the program's website.

For MPS II, a patient must:

- satisfy the initial and ongoing eligibility criteria as detailed in these guidelines
- participate in the evaluation of effectiveness of the drug 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 II, that might compromise the effectiveness of the drug 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 idursulfase, for the treatment of MPS II through the LSDP:

- Patients with the presence of another life threatening or severe disease where the long term prognosis is unlikely to be influenced by enzyme replacement therapy (ERT).
- The presence of another medical condition that might reasonably be expected to compromise a response to ERT.

Initial eligibility requirements

Diagnosis

The diagnosis of MPS II must be confirmed by the demonstration of a deficiency of iduronate 2-sulfatase in white blood cells with the assay performed in a NATA-accredited laboratory; or for siblings of a known patient, detection of a disease-causing mutation.

A deficiency of iduronate 2-sulfatase in white blood cells should be confirmed by either an enzyme assay in cultured skin fibroblasts or by detection of a disease-causing mutation in the iduronate 2-sulfatase gene.

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

- **Sleep disordered breathing:** Patients with an Apnoea/Hypopnoea Incidence of >5 events/hour of total sleep time or more than 2 severe episodes of desaturation (oxygen saturation <80%) in an overnight sleep study.
- **Respiratory function tests:** Patients with FVC less than 80% of predicted value for height.
- **Cardiac:** Myocardial dysfunction as indicated by a reduction in ejection fraction to less than 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 greater than 10 degrees from normal in shoulders, neck, hips, knees, elbows or hands.
- **Infants and children aged less than 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 II, for example by genotyping, with clear prediction of progress of the disease, or if, on the basis of a sibling's disease progression, severe disease can be predicted.

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.

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 II must be provided.

At the time of onset of treatment, clinicians and patients should jointly agree on criteria for cessation of the ERT using indicators that reflect lack of response to therapy or deterioration in somatic or neurological disease despite therapy.

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.

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 II, evidencing the effectiveness of the therapy
- 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 the infusion rate
- 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; or
- 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.

See the reapplication form for existing patients.

Patients who are applying to recommence treatment following a break should use the reapplication form.