Australian Government
Department of Health and Aged Care

Guidelines for the treatment of mucopolysaccharidosis type IVA (MPS IVA) 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 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 IVA 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 IVA through the LSDP

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

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

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

The generic name for this drug is elosulfase alfa. The trade name for this drug is Vimizim®.

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

Dosage

The recommended dosage for elosulfase alfa is 2 mg/kg of body weight adminstered once a week.

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 IVA through the LSDP.

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

For MPS IVA, 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 undergoing assessments, 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 IVA, that might compromise the effectiveness of the drug treatment
- 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 elosulfase alfa, for the treatment of MPS IVA through the LSDP:

- A lung capacity (FVC) of less than 0.3 litres and requiring ventilator assistance.
- 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 IVA must be confirmed by the following tests:

1. elevated urine mucopolysaccharide substrate (uMPSs) analysis at baseline
2. deficiency of N-acetylgalactosamine-6-sulfatase (GALNS) in white blood cells or skin fibroblasts.

Genetic Testing

Provisional eligibility for the program will be accorded on the basis of elevated uMPSs and GALNS deficiency. Genetic testing for mutations in the GALNS gene is to be performed in a NATA-accredited laboratory for confirmation of diagnosis of MPS IVA within 6 months of commencement of acceptance into the program, with the results reported to the LSDP. If 2 pathogenic mutations are not confirmed by genetic testing, a review of the eligibility based on the original uMPSs quantitative analysis and GALNS deficiency data may be undertaken by the LSDP in consultation with one or more expert clinicians/scientists who have experience and expertise in MPS IVA. It is accepted that, currently, not all individuals with MPS IVA will have 2 pathogenic mutations detected and strong evidence of elevated uMPSs and GALNS deficiency may be accepted as long-term eligibility criteria after receipt of the genetic testing report at the 6 month review point.

Demonstration of initial response to treatment

Provisional eligibility for the program will be accorded on the basis of elevated uMPSs at baseline (and GALNS deficiency). uMPSs quantitative analysis is to be provided at the following time points:

1. diagnosis (to establish a baseline)
2. 1 to 3 months post initiation to treatment
3. if a 20% reduction is not shown at (2) then at 6 months post initiation to treatment.

For patients who do not demonstrate the 20% reduction from baseline by 6 months post-treatment initiation, the treating physician will be required to submit a clinic letter providing alternative measures to demonstrate how the patient is responding to therapy, including any relevant documentation, for the

LSDP's assessment. If an alternative test is used to demonstrate a response to therapy, baseline and post-treatment results for the alternative test must be provided to the LSDP.

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. It is the expectation of the LSDP that patient management will be consistent with clinical guidelines for MPS IVA.

Treating physicians will be required to submit a clinic letter outlining the patient's recent medical/surgical history, general description of their health status and indication that the patient is responding to treatment. It is a clinical decision as to which tests are undertaken annually to monitor patients. Any relevant test results should be outlined in the clinic letter.

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:

- a patient is unable to demonstrate a 20% reduction in their baseline uMPSs analysis by 6 months post initiation to treatment, and the treating physician has not provided adequate alternative documentation to demonstrate a response to therapy (as outlined under the Initial eligibility requirements above). Please note that uMPSs analysis is not required to be provided to the LSDP on a regular basis once the 20% reduction from baseline has been demonstrated. Patients who have been approved to receive subsidised therapy prior to December 2023 will no longer be required to provide uKS or uMPSs analyses
- 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
- 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 the sponsor 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.