



Guidelines for the treatment of acid sphingomyelinase deficiency (ASMD) type A/B and type B 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 ASMD type A/B or type B 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 ASMD type A/B and type B through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of ASMD type A/B or type B.

Medicines currently available for the treatment of ASMD type A/B and type B through the LSDP

There is one medicine currently subsidised through the LSDP for the treatment of ASMD type A/B and type B.

The generic name for this medicine is olipudase alfa. The trade name for this medicine is Xenpozyme®.

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

Dosage

Refer to the product information for guidance on the recommended dose. When initiating treatment with olipudase alfa, or restarting after a treatment break, the dose is escalated. Patients are then treated with a maintenance dose.

The maximum dosage of olipudase alfa that is subsidised through the LSDP is 3mg/kg per fortnight.

Home infusion

For patients receiving olipudase alfa, home administration by a trained health care professional may be considered for individual patients on the maintenance dose after safety and tolerability has been established in the clinical setting. Requests should be made by the patient's treating physician, or an appropriate member of the patient's clinical care team, directly to the sponsor.

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 ASMD type A/B and type B through the LSDP.

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

For ASMD type A/B and type B, 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 ASMD type A/B and type B, that might compromise the effectiveness of the medicine 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 olipudase alfa for the treatment of ASMD type A/B and type B through the LSDP:

- Patients with ASMD Type A.
- Patients with potentially confounding diagnoses, such as Gaucher disease.

- Patients with another life threatening or severe disease where the long-term prognosis is unlikely to be influenced by therapy.
- Patients with another medical condition that might reasonably be expected to compromise a response to therapy.

Initial eligibility requirements

Diagnosis

The diagnosis of ASMD type A/B and type B must be confirmed by using one of the following methods:

1. Documented deficiency of acid sphingomyelinase and normal glucocerebrosidase activity in any of peripheral blood leucocytes, fibroblasts, or lymphocytes;

OR

2. Documented molecular genetic testing indicating biallelic disease-causing variants in the sphingomyelin phosphodiesterase 1 gene (SMPD1).

Note: While the LSDP requires only one of the above tests to confirm diagnosis for program eligibility, results of both tests should be submitted if available.

Eligibility criteria

The patient must also satisfy the following criteria to be eligible for treatment with olipudase alfa:

1. A spleen volume at least 5 times normal volume based on age, gender and body habitus, as relevant, or the patient has undergone splenectomy;

AND EITHER

- Interstitial lung disease as demonstrated by imaging and/or a DLco <70% predicted;

Notes:

- In children under six, only imaging is required.
- Patients who have undergone a lung transplant as a consequence of ASMD disease progression are considered to have met the interstitial lung disease criterion, where documentation of the lung transplant is provided.

OR

3. Clinically significant non-transient sequelae of organ damage (e.g. liver enzymes greater than twice the upper limit of normal, pain, symptoms related to hypersplenism).

Ongoing eligibility requirements

The treating physician must submit a 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 ASMD type A/B and type B must be provided.

The treating physician must declare the patient continues to meet the eligibility criteria to receive subsidised treatment through the LSDP in accordance with the 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 the test results and the Excel spreadsheet for ASMD type A/B and type B, 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 the symptoms of disease that originally resulted in the patient being approved for subsidised treatment
- the patient experiences unresolved severe injection site or severe hypersensitivity reactions
- the patient develops another life threatening or severe disease where the long-term prognosis is unlikely to be influenced by treatment
- the patient develops another medical condition that might reasonably be expected to compromise a response to treatment
- 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.