Australian Government
Department of Health and Aged Care

Guidelines for the treatment of infantile-onset lysosomal acid lipase deficiency (LAL-D) 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 infantile-onset LAL-D 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 infantile-onset LAL-D through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of infantile-onset LAL-D.

Medicines currently available for the treatment of LAL-D through the LSDP

There is one medicine currently subsidised through the LSDP for the treatment of infantile-onset LAL-D.

The generic name for this medicine is sebelipase alfa.The trade name for this medicine is Kanuma®.

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

Dosage

The maximum dosage of sebelipase alfa that is subsidised through the LSDP is 5mg/kg/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 infantile-onset LAL-D through the LSDP.

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

For infantile-onset LAL-D, 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 infantile-onset LAL-D, 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 sebelipase alfa for the treatment of infantile-onset LAL-D through the LSDP:

- 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.
- Patients requiring liver transplantation.

Initial eligibility requirements

Diagnosis

The diagnosis of infantile-onset LAL-D must be confirmed by evidence demonstrating:

1. No detectable or severe deficiency in LAL enzyme activity when tested at the National Referral Laboratory; AND
2. *LAL (LIPA)* mutations on genetic testing (noting treatment may commence prior to the results of the genetic test being available if necessary).

Diagnosis of infantile-onset LAL-D must be confirmed when the patient is under 12 months of age.

Initial treatment may commence based on the results of the LAL enzyme activity test. Within 8 weeks of commencing treatment, results of the outstanding infantile-onset LAL-D disease genetic test must be forwarded to the LSDP for the patient to continue ongoing subsidised treatment through the LSDP.

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 infantile-onset LAL-D 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 infantile-onset LAL-D, 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 prevent significant morbidity due to infantile-onset LAL-D. Significant morbidity may include (but is not limited to) failure to thrive, liver failure, adrenal failure, and severe nutritional deficiency
- the patient experiences unresolved severe injection site or severe hypersensitivity reactions
- 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.