Australian Government
Department of Health and Aged Care

Guidelines for the treatment of late-infantile onset Batten disease (CLN2 disease) 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 CLN2 disease 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 CLN2 disease through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of CLN2 disease.

Drugs currently available for the treatment of CLN2 disease through the LSDP

There is one drug currently subsidised through the LSDP for the treatment of CLN2 disease.

The generic name for this drug is cerliponase alfa. The trade name for this drug is Brineura®.

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

Dosage

The maximum dosage of cerliponase alfa that is subsidised through the LSDP is outlined in Table 1. It is administered by infusion via the intracerebroventricular route every other week.

Table 1: Dose and volume of cerliponase alfa based on cerliponase alfa (Brineura®) 30 mg/mL solution for injection.

Age Groups	Total dose administered every other week (mg)	Volume of Brineura solution (mL)
Birth to <6 months	100	3.3
6 months to <1 year	150	5
1 year to <2 years	200 (first 4 doses) 300 (subsequent doses)	6.7 (first 4 doses) 10 (subsequent doses)
2 years and older	300	10

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 CLN2 disease through the LSDP.

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

For CLN2 disease, 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 CLN2 disease, 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 cerliponase alfa, for the treatment of CLN2 disease through the LSDP:

- Patients who fail to attend their clinic for assessments twice a year, and do not have an acceptable reason to do so.
- 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.

Note: There is no evidence of treatment benefit when patients reach an unreversed motor language (ML) score of zero (0).

Initial eligibility requirements

Diagnosis

The diagnosis of CLN2 disease must be confirmed by the demonstration of the following tests:

- Tripeptidylpeptidase 1 (TPP1) enzyme deficiency: TPP1 deficiency in white blood cells or skin fibroblasts, and
- Genetic test for mutations of the CLN2 gene: Genetic testing for mutations in the CLN2 gene shall be performed to confirm the diagnosis of CLN2 disease.

Initial treatment may commence based on the presence of TPP1 deficiency. Within 8 weeks of commencing initial treatment, results of the CLN2 disease genetic test must be forwarded to the LSDP to be considered for ongoing subsidised treatment though the LSDP.

If 2 pathogenic mutations are not confirmed by genetic testing a review of the eligibility based on the TPP1 deficiency data and genetic test results may be undertaken by the LSDP in consultation with one or more expert clinicians who have experience and expertise in CLN2 disease. In such cases, strong evidence of TPP1 deficiency may be accepted as long term eligibility criteria only after receipt of the genetic test results.

Baseline results for complications of CLN2 disease are to be provided with supporting evidence which may include but is not limited to language ability, motor ability, ML score, feeding status, seizures, myoclonus and vision.

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 stabilisation or slowing of progression of the patient's condition, and evidence to support ongoing eligibility for the treatment of CLN2 disease 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 the 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 the test results and the Excel spreadsheet for CLN2 disease evidencing the effectiveness of the therapy
- failure to attend clinic appointments for assessments twice a year, or have an unacceptable reason not to participate

- the patient has severe infusion related reactions which are not preventable/manageable 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.

Physicians are required to inform the LSDP if patients demonstrate a sustained decline ≥2 points in the ML score over any continuous 48 week period during their course of treatment.

Note:

- There is no evidence to support benefits from ongoing treatment with cerliponase alfa for patients who have experienced a decline of ≥2 points in the ML score over any continuous 48 week period during their course of treatment.
- There is no evidence of clinical or quality of life benefit to patients with a ML score of zero (0). Therefore, clinicians and families should consider the risk to benefit ratio and give serious consideration to ceasing treatment when a patient reaches an irreversible ML score of zero (0).

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