



# Guidelines for the treatment of perinatal- or infantile-onset hypophosphatasia (HPP) 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 perinatal- or infantile-onset HPP 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 perinatal- or infantile-onset HPP through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of perinatal- or infantile-onset HPP.

## Drugs currently available for the treatment of perinatal- or infantile-onset HPP through the LSDP

There is one drug currently subsidised through the LSDP for the treatment of perinatal- or infantile-onset HPP.

The generic name for this drug is asfotase alfa.

The trade name for this drug is Strensiq®.

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

## Dosage

The maximum dosage of asfotase alfa that is subsidised through the LSDP is 2mg/kg of body weight, administered 3 times per week; or 1 mg/kg of body weight, administered 6 times per 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 perinatal- or infantile-onset HPP through the LSDP.

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

For perinatal- or infantile-onset HPP, 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 of perinatal- or infantile-onset HPP, 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 asfotase alfa for the treatment of perinatal- or infantile-onset HPP through the LSDP:

- Patients with another life-threatening condition where prognosis is unlikely to be influenced by an enzyme replacement therapy (ERT)
- Patients with another medical condition(s) that might reasonably be expected to compromise a response to an ERT.

## Initial eligibility requirements

### Diagnosis

The diagnosis of perinatal- or infantile-onset HPP must be confirmed by:

- Alkaline phosphatase (ALP) activity below the lower limit of normal (age- and sex-adjusted)
- Exclusion of non-HPP-related causes of low ALP activity or confirmation of HPP via pyridoxal 5'-phosphate (PLP) and/or urine phosphoethanolamine (PEA) and/or genetic testing
- Paediatric medical records documenting HPP-related symptoms
- History of HPP-related bone disease, as assessed by skeletal imaging (radiography).

The patient must satisfy the following criterion to be eligible for treatment with asfotase alfa:

- Symptomatic perinatal- or infantile-onset HPP in patients at time of the initial LSDP application, with a history of any of the disease manifestations listed below:
  - Respiratory compromise requiring ventilatory support or
  - Vitamin B6-dependent seizures or
  - Rachitic chest deformity.

## 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 perinatal- or infantile-onset HPP 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 perinatal- or infantile-onset HPP, evidencing the effectiveness of the therapy
- 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 LSDP subsidised treatment
- the patient develops another medical condition that might reasonably be expected to compromise a response to LSDP subsidised 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.

See the [reapplication form for existing patients](#).

Patients who are applying to recommence treatment following a break should use the [reapplication form](#).