Guidelines for the treatment of Fabry disease through the Life Saving Drugs Program

The 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 Australian Government–subsidised treatment for Fabry disease through the LSDP.

It describes the criteria for general and ongoing eligibility to access subsidised treatment and the administrative requirements associated with annual reapplications.

Treatment of Fabry disease through the LSDP

From 1 August 2026 Australian Government–subsidised drug treatment is available only for patients with Fabry disease receiving treatment through the LSDP from up to and including 31 July 2026 who continue to meet all eligibility criteria.

Note: pegunigalsidase alfa (Elfabrio®) for the treatment of Fabry disease is available through the Pharmaceutical Benefits Scheme (PBS) from 1 August 2026.

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

There are 2 drugs currently subsidised through the LSDP for the ongoing treatment of Fabry disease.

The generic names for these drugs are agalsidase alfa and agalsidase beta.

The trade names for these drugs are Replagal® and Fabrazyme®.

The Therapeutic Goods Administration (TGA) registration and Product Information for agalsidase alfa (Replagal®) and agalsidase beta (Fabrazyme®) can be found on the TGA's website.

Choice of treatment

Treating physicians can request the most appropriate drug to treat their patient.

All patients who are initiated on a drug or transitioned to a different drug through the LSDP are required to remain on the same drug for a period of at least 12 months, unless there is objective clinical evidence of ongoing clinical deterioration or significant adverse reactions.

Dosage

The maximum dosage of agalsidase alfa that is subsidised through the LSDP is 0.2 mg/kg per fortnight.

The maximum dosage of agalsidase beta that is subsidised through the LSDP is 1 mg/kg per fortnight.

Home infusion

If a patient wishes to receive and is assessed to be suitable for treatment through a home infusion service, the patient must have received at least 12 infusions in the hospital setting and have been assessed by the treating physician as medically stable, meaning that any infusion associated reactions are well controlled. Home infusion programs are provided by the pharmaceutical sponsors of agalsidase alfa and beta and are not part of the LSDP.

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 Fabry disease through the LSDP.

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

For Fabry 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 Fabry disease, that might compromise the effectiveness of the drug treatment
- be an Australian citizen or permanent Australian resident who qualifies for Medicare.

Exclusion criteria

The following patients are not eligible for subsidised treatment with agalsidase alfa or agalsidase beta for the treatment of Fabry disease through the LSDP:

- Patients with related Fabry disease conditions which may compromise response to Enzyme Replacement Therapy (ERT).
- Patients with a presence of another life-threatening or severe disease where the long term prognosis is unlikely to be influenced by ERT.
- The presence of another medical condition that might reasonably be expected to compromise a response to ERT.
- Receiving treatment with an ERT for Fabry disease through the PBS.

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.

Initial eligibility requirements

Patients initiating on ERT for the treatment of Fabry disease can initiate with peguligalisase alfa on the PBS from 1 August 2026. New patients will not be commenced under the LSDP from 1 August 2026.

Diagnosis

The diagnosis of Fabry disease must be confirmed by demonstration of specific deficiency of alpha-galactosidase enzyme activity in blood or white cells or by the presence of genetic mutations known to result in deficiency of alpha- galactosidase enzyme activity.

Patients must satisfy at least one of the following criteria to be eligible for treatment with agalsidase alfa or agalsidase beta:

a) Fabry-related renal disease

Confirmation by renal biopsy is recommended for all patients to:

- provide prognostic information
- exclude other causes of nephropathy
- demonstrate evidence of focal glomerular sclerosis or fibrosis greater than that expected for age, once other causes of nephropathy have been excluded
- document significant histological changes related to Fabry disease.

Male Fabry patients:

- abnormal albumin (>20 µg/min), as determined by 2 separate samples, at least 24 hours apart; and/or
- abnormal protein excretion (>150 mg/24 hours); and/or
- albumin: creatinine ratio greater than upper limit of normal, in 2 separate samples, at least 24 hours apart; and/or
- renal disease due to long-term accumulation of glycosphingolipids in the kidneys.

Female Fabry patients:

- proteinuria >300 mg/24 hours with clinical evidence of progression.
- renal disease due to long-term accumulation of glycosphingolipids in the kidneys.

b) Fabry-related cardiac disease

Confirmation by myocardial biopsy is recommended to exclude other causes of cardiac hypertrophy.

Left ventricular hypertrophy, as evidenced by cardiac MRI or echocardiogram data, in the absence of hypertension. If hypertension is present, it should be treated optimally for at least 6 months prior to the submission of an application through this criterion.

Significant life-threatening arrhythmia or conduction defect.

c) Ischaemic vascular disease

Shown on objective testing with no other cause or risk factors identified.

d) Uncontrolled chronic pain

Uncontrolled chronic pain despite the use of maximum doses of appropriate analgesia and antiepileptic medications for peripheral neuropathy. Patients meeting this criterion must provide ongoing evidence of effect, through analgesic intake, pain diary, summary letter from treating physician.

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 Fabry 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 Fabry disease, 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 has severe infusion-related adverse reactions which are not preventable by appropriate pre-medication and/or adjustment of infusion rates
- 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.