



# 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, initial and ongoing eligibility to access subsidised treatment and the administrative requirements associated with the initial application and annual reapplications.

### Treatment of Fabry disease through the LSDP

Australian Government–subsidised drug treatment is available for eligible patients with a confirmed diagnosis of Fabry disease.

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

There are 3 drugs currently subsidised through the LSDP for the treatment of Fabry disease.

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

The trade names for these drugs are Replagal<sup>®</sup>, Fabrazyme<sup>®</sup> and Galafold<sup>®</sup>.

The Therapeutic Goods Administration (TGA) registration and Product Information for agalsidase alfa (Replagal<sup>®</sup>), agalsidase beta (Fabrazyme<sup>®</sup>) and migalastat (Galafold<sup>®</sup>) can be found on the [TGA's website](#).

### Choice of treatment

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

Migalastat can only be used for patients who have been treated with agalsidase alfa or agalsidase beta for at least 12 months.

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.

The maximum dosage of migalastat that is subsidised through the LSDP is 150 mg every second day.

## 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.

## 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, agalsidase beta or migalastat, for the treatment of Fabry disease through the LSDP:

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

Patients participating in a clinical trial are not eligible for subsidised treatment through the LSDP. Previous involvement in a clinical trial does not impede eligibility to subsidised treatment through the LSDP.

In addition to the above, patients treated with migalastat must not be younger than 16 years or have severe renal insufficiency, defined as estimated GFR less than 30 mL/min/1.73m<sup>2</sup>.

## Initial eligibility requirements

### 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, agalsidase beta or migalastat:

#### 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.

## Migalastat

For treatment with migalastat, patients must meet the above criteria as well as have an amenable genetic mutation. To determine if a patient's mutation is amenable to treatment with migalastat, search the [Galafold Amenability Table](#) (GAT). For questions about the amenability status of a mutation or the website noted above, contact Amicus Australia's Medical Information Department on [MedInfoAustralia@amicusrx.com](mailto:MedInfoAustralia@amicusrx.com).

Patients must have been treated with agalsidase alfa or agalsidase beta for at least 12 months or must be intolerant to agalsidase alfa or agalsidase beta.

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 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 and has a non-amenable mutation to migalastat
- 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 due to participation in a clinical trial or any other reason should use the [initial application form](#).