



New arrangements for migalastat (Galafold[®]) – information for prescribers

August 2024

Migalastat (Galafold[®]) for Fabry disease is moving to the Pharmaceutical Benefits Scheme (PBS).

From 1 September 2024, following a recommendation by the Pharmaceutical Benefits Advisory Committee (PBAC) at its [March](#) and [May](#) 2024 meetings, migalastat (Galafold[®]) will be available through the PBS under the General Schedule, rather than the Life Saving Drugs Program (LSDP).

There is no change to arrangements for enzyme replacement therapies (ERT) for Fabry disease (agalsidase alfa (Replagal[®]) and agalsidase beta (Fabrazyme[®])). These medicines will continue to be accessed through the LSDP.

How will accessing migalastat (Galafold[®]) change?

From 1 September 2024, access to migalastat (Galafold[®]) will be through the PBS. A completed authority application form for Fabry disease will be required to transition patients to the PBS. Written authority applications can be made in writing to Services Australia via mail/postal service or through Health Professional Online Services (HPOS) upload, for new patients and patients transitioning from the LSDP.

Services Australia aims to process written authority requests within an average of 1 to 2 weeks from when Services Australia receives the authority application. Processing times may vary when volumes are high or when information in the application is not complete. Additional postage time applies to mailed authority applications and this is outside Services Australia's control. Postage delays can be prevented if prescribers choose to upload the application via the Health Professional Online Services at www.servicesaustralia.gov.au/hpos

Authority requests for continuing treatment may be requested electronically, in real-time through the Online PBS Authorities System or by telephone by contacting Services Australia on 1800 700 270.

Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270. Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at <https://www.servicesaustralia.gov.au/>

Who can prescribe migalastat (Galafold[®])?

Migalastat (Galafold[®]) can only be prescribed by a physician with expertise in the management of Fabry disease.

When does LSDP supply cease?

For LSDP patients currently taking migalastat (Galafold®), LSDP supply will cease on 1 December 2024. This will allow existing patients 3 months (from 1 September 2024 to 30 November 2024) to transition to accessing their subsidised supply under the PBS. We recommend that you seek to transition your patient as soon as the PBS listing is available (from 1 September 2024) to ensure they receive authority approval to access their medication on time without impacting their treatment regimen.

The LSDP will not approve access to migalastat (Galafold®) for any new patients from 1 September 2024.

Will patient access to migalastat (Galafold®) be impacted by this new arrangement?

There should be no impact to any eligible patients' access and continuing treatment.

For existing patients currently accessing treatment under the LSDP:

To ensure a smooth transition we recommend prescribers schedule an appointment with patients who are currently taking migalastat (Galafold®) as soon as possible after 1 September 2024 to apply for a prescription under the PBS.

Until 30 November 2024, the LSDP will ensure that nominated authorised pharmacies can continue to access treatment for existing patients to provide time for them to transition to the PBS. Once patients have their approved authority prescription, their supply under the LSDP will cease.

A co-payment will apply for access to migalastat (Galafold®) through the PBS, in line with other PBS medications.

For new patients who require access to migalastat prior to 1 September 2024:

Prior to 1 September 2024, treating physicians can apply for access through the LSDP for patients who meet LSDP eligibility requirements. From 1 September 2024, the LSDP will no longer accept any applications for treatment with migalastat (Galafold®), including transition of current LSDP patients receiving ERT.

Please note that access to migalastat (Galafold®) through the LSDP requires completion of 12 months treatment with ERT. This requirement will not apply for access to migalastat (Galafold®) through the PBS. However, it cannot be waived for access through the LSDP prior to 1 September 2024.

For new patients who require access to migalastat after 1 September 2024:

You will need to follow the PBS written authority approval process ensuring that new patients meet all the PBS eligibility criteria.

How often will I need to see my patient to renew their script?

Under the LSDP, medical practitioners must apply annually on behalf of patients to obtain access to migalastat. The frequency of prescription application will now be up to 6-monthly depending on the treatment phase as set out in the PBS Schedule for migalastat.

Are the eligibility criteria for PBS access to migalastat (Galafold®) the same as the LSDP criteria?

The PBS restriction for access to migalastat (Galafold®) is similar to the LSDP eligibility criteria. The main differences are:

- New patients will not be required to complete 12 months treatment with ERT to be eligible to access migalastat (Galafold®) through the PBS. Migalastat (Galafold®) will be available as a first line therapy for amenable patients.
- The age eligibility for patients will be changed from '16 years or older' to '12 years or older'.

What information do medical practitioners have to provide when seeking the PBS authority approval?

Patients must have a diagnosis of Fabry disease and have a documented migalastat (Galafold®) amenable galactosidase alpha (GLA) gene variant.

Prescribers must ensure that their patient meets the PBS eligibility criteria, documenting the diagnostic reports, including the confirmed mutations, in the patient's medical records and completing the relevant authority application form for Fabry disease on the Services Australia website at www.servicesaustralia.gov.au.

The PBS listing of migalastat (Galafold®) (PBS item code 14573B) includes an initial treatment phase for new patients, a grandfather arrangement to allow transitioning of patients from LSDP funded therapies, and a continuing treatment phase for patients meeting the continuing treatment eligibility criteria who wish to continue migalastat (Galafold®) therapy through the PBS. Eligibility criteria are explained in detail below.

New patients can access PBS subsidised treatment through the initial treatment restrictions. Eligibility criteria that must be documented in the patient's medical records include:

- Patients must have at least one of (i) documented deficiency of alpha- galactosidase enzyme activity in blood (ii) presence of genetic mutations known to result in deficiency of alpha-galactosidase enzyme activity; **and**
- Patient must have a documented migalastat amenable galactosidase alpha (GLA) gene variant; **and**
- Patient must have an estimated glomerular filtration rate (eGFR) of at least 30 mL/min/1.73 m²; **and**

- Patient must be male with Fabry-related renal disease confirmed by at least one of the following: (i) abnormal albuminuria of more than 20 mcg/min), as determined by 2 separate samples at least 24 hours apart, (ii) abnormal proteinuria of more than 150 mg/24 hours), (iii) albumin: creatinine ratio greater than upper limit of normal in 2 separate samples at least 24 hours apart, (iv) renal disease due to long-term accumulation of glycosphingolipids in the kidneys, **OR**
- Patient must be female with Fabry-related renal disease confirmed by at least one of the following: (i) proteinuria of more than 300 mg/24 hours with clinical evidence of progression, (ii) renal disease due to long-term accumulation of glycosphingolipids in the kidneys, **OR**
- Patient must have Fabry-related cardiac disease confirmed by at least one of the following: (i) Left ventricular hypertrophy, as evidenced by cardiac magnetic resonance imaging (MRI) or echocardiogram data, in the absence of hypertension, (ii) Significant life-threatening arrhythmia or conduction defect, (iii) Late gadolinium enhancement or a low T1 on cardiac MRI, **OR**
- Patient must have Fabry-related either (i) ischaemic disease, (ii) cerebrovascular disease as shown on objective testing with no other cause or risk factors identified, **OR**
- Patient must have Fabry-related uncontrolled chronic pain despite the use of recommended doses of appropriate analgesia and antiepileptic medications for peripheral neuropathy, **OR**
- Patient must have significant Fabry-related gastrointestinal symptoms despite the use of the recommended doses of appropriate pharmacological therapies.

Patients who are transitioning from LSDP-funded Fabry disease therapy can access PBS subsidised treatment through ‘the grandfather arrangement’ restriction. Prescribers will assess eligibility and document the following information in the patient’s medical records:

- Patient must have a documented migalastat amenable galactosidase alpha (GLA) gene variant prior to commencing treatment with migalastat; and
- Patient must have/ have had an estimated glomerular filtration rate (eGFR) of at least 30 mL/min/1.73 m² prior to commencing treatment with migalastat.

Can patients receive continuing treatment?

Patients can receive continuing treatment with migalastat by qualifying under the continuation treatment restriction criteria, which includes:

- Patient must have received prior PBS-subsidised treatment with this drug for this condition; **and**
- Patient must have demonstrated clinical improvement or stabilisation of condition, the details of which must be kept with the patient’s record; **and**
- Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by migalastat.

From 1 September 2024, you can refer to the [PBS schedule](#) for further details.

Is there a change to the cost for patients?

Yes. Like other medicines subsidised under the PBS, patients are required to make a co-payment towards the cost of their treatment with migalastat (Galafold®). Current co-payments (in 2024) are \$31.60 (\$7.70 with a concession card), per dispensing of each prescription.

The patient contribution towards PBS prescriptions for migalastat (Galafold®) will also contribute towards the patient's [PBS Safety Net](#) threshold.

For further information, patients may contact their local pharmacy, call the PBS Information Line on 1800 020 613 or visit the PBS website at www.pbs.gov.au or Services Australia website at www.servicesaustralia.gov.au.

Will pharmacies stock these medicines?

Yes. However, some pharmacies may only stock certain medicines on an as needed or individual patient basis. We recommend that patients contact their chosen pharmacy to confirm availability and ongoing supply arrangements.

What information or support is available for my patients?

Patients can access a Factsheet on the changes to accessing migalastat (Galafold®) on the PBS at www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/for-patients.

Patients can also seek further advice from their pharmacist.