5.28 AGALSIDASE ALFA, Solution for I.V. injection 3.5 mg in 3.5 mL vial, Replagal®, Takeda Pharmaceuticals Australia Pty Ltd

AGALSIDASE BETA, Powder for IV infusion, 5 mg vial, Powder for IV infusion, 35 mg vial, Fabrazyme®, Sanofi Australia and New Zealand

1 Purpose of Submission

1.1 The PBAC was requested to consider the suitability of the enzyme replacement therapies (ERT) agalsidase alfa (Replagal®) and agalsidase beta (Fabrazyme®) for PBS listing for the treatment of patients with Fabry disease (FD), under the same conditions it recommended for the listing of pegunigalsidase alfa (Elfabrio®) for the treatment of FD.

2 Background

2.1 Agalsidase alfa was considered by the PBAC at its September 2002 and September 2003 meetings. Agalsidase beta was considered by the PBAC at its June 2002 and December 2002 meetings.

2.2 In line with PBAC and LSDP operations at the time, the PBAC determined at its December 2002 meeting that agalsidase beta, and at its September 2003 meeting that agalsidase alfa, met the LSDP listing requirements and could be considered by government for inclusion on the LSDP.

2.3 Agalsidase alfa and agalsidase beta have been available through the LSDP since July 2004.

2.4 At its November 2009 meeting the PBAC was asked to consider a report on the updated literature review of the comparative efficacy and safety of agalsidase alfa versus agalsidase beta, to determine whether the pricing disparity in the average yearly patient

treatment cost at the registered doses between these agents was justified. The PBAC recommended that the price of agalsidase beta be reduced to meet the cost on a per patient basis of agalsidase alfa on the basis that there was insufficient evidence at that stage to support a finding that there is any clinical difference between the alfa and beta forms at the registered doses (Section 8, updated literature review of agalsidase alfa and agalsidase beta for the treatment of Fabry disease Public Summary Document [PSD], November 2009 PBAC meeting).

2.5 The LSDP EP undertook a review of agalsidase alfa and beta in 2018 and similarly determined that there was insufficient evidence to support any claim of a clinically significant difference between agalsidase alfa and beta.

2.6 At its June 2022 LSDP EP meeting the LSDP EP considered evidence from multiple peer reviewed publications, as detailed in the Drug Utilisation Sub Committee (DUSC) review and the Fabry Australia Medical Advisory Committee White Paper on the current prevalence of FD. On the basis of the evidence before it the LSDP EP determined that FD no longer met the LSDP's ultra-rare disease criterion of less than 1 in 50,000 people.

2.7 The PBAC executive (Chair and Deputy Chair) attended the June 2022 LSDP EP meeting for this item. The PBAC Chair advised that, noting the sponsor of migalastat (Galafold®) was seeking an amendment to its current listing, it would be easier to transition migalastat to the PBS first and then allow the remaining FD medicines to follow Following the LSDP EP referral the PBAC considered migalastat at its December 2022 and March 2024 meetings. Migalastat was recommended by the PBAC at its March 2024 meeting and listed on the PBS in September 2024 with its subsequent delisting from the LSDP following a three month transition period. Migalastat is an oral alternative to ERTs, which is PBS listed for people 12 years and older with a confirmed diagnosis of FD and who have an amenable mutation.

2.8 The inclusion of migalastat on the PBS added a further layer of ineligibility to the provision of FD treatments remaining funded through the LSDP. Medicines are not eligible for inclusion on the LSDP if there is a cost-effective alternative available on the PBS. However, as only patients with amenable mutations would benefit from treatment with migalastat, subsidy of the ERTs remained through the LSDP.

2.9 At its July 2025 meeting the PBAC recommended the listing of pegunigalsidase alfa (Elfabrio®), an ERT, for the treatment of FD. At time of consideration the PBAC considered that while the other ERTs would be appropriate clinical comparators, as they are not listed on the PBS, PBS-listed migalastat is an appropriate comparator (Paragraph 7.7, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

2.10 The PBAC considered pegunigalsidase alfa to have non-inferior comparative efficacy and safety to migalastat, and recommended listing pegunigalsidase alfa on a cost-minimisation basis to migalastat (Paragraph 7.8, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

2.11 At its July 2025 meeting, the PBAC also determined that the claim that pegunigalsidase alfa has non-inferior efficacy compared to agalsidase beta was adequately supported. The PBAC considered there was a lack of clinical evidence to support the claim of non-inferior efficacy compared to agalsidase alfa, however it was reasonable to conclude that agalsidase alfa has similar efficacy to agalsidase beta, and therefore pegunigalsidase alfa (Paragraph 7.4 pegunigalsidase alfa PSD, July 2025 PBAC meeting).

2.12 PBAC consideration of pegunigalsidase alfa has hence provided a cost-effective price for agalsidase alfa and beta.

2.13 The PBS listing of pegunigalsidase alfa would provide a clinically effective and cost-effective alternative to the non-cost-effective LSDP listed agalsidase alfa or agalsidase beta and would therefore result in the discontinuation of these medicines subsidy via the LSDP, with LSDP patients likely transitioning to an available PBS listed FD ERT.

Registration status

2.14 Agalsidase alfa was TGA registered on 17 May 2002 for long-term enzyme replacement therapy of patients with Fabry Disease (alpha-galactosidase A deficiency).

2.15 Agalsidase beta was TGA registered on 30 May 2002 for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (alpha-galactosidase deficiency).

3 Proposed listing

3.1 At is July 2025 meeting the PBAC recommended the listing of pegunigalsidase alfa for the treatment of FD as an Authority Required (Written) listing for initial treatment and an Authority Required (telephone/electronic) listing for continuing treatment, on the basis that it should be available only under Section 100 (Highly Specialised Drugs Program (Public and Private)) (Paragraph 7.1, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

3.2 The proposed listings for agalsidase alfa and agalsidase beta are to align with the PBAC's recommended listing of pegunigalsidase alfa for FD including a grandfather clause to allow patients who are currently treated with LSDP funded agalsidase alfa and agalsidase beta to transition to the PBS-subsidised treatment. The proposed listings included minor

amendments to the restriction wordings for the 'switching treatment phase' to allow switching from any of the PBS listed treatments for FD to agalsidase alfa or agalsidase beta. The PBAC is asked if this is appropriate. Additionally, if recommended, the PBAC is asked if it is appropriate for the same changes to flow-on to the PBS listing of migalastat and the recommended listing of pegunigalsidase alfa when listed to allow patients to switch between the PBS-subsidised treatments for FD.

MEDICINAL PRODUCT **medicinal product pack**	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
AGALSIDASE ALFA					
agalsidase alfa 3.5 mg/3.5 mL injection, 3.5 mL vial	NEW HSD public NEW HSD private	1	1	5	Replagal

	Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)
	Prescriber type: ☒ Medical Practitioners
	Benefit type: ☒ Authority Required (FULL assessment) in writing via OPA/post/HPOS upload)
	Authority type: ☒ Complex Authority Required (CAR)
	Prescribing rule level:
	Administrative Advice: No increase in the maximum number of repeats may be authorised.
	Administrative Advice: Special Pricing Arrangements apply.
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Initial treatment
	Clinical criteria:
	Patient 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
	Clinical criteria:
	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 antiseizure medications for peripheral neuropathy; OR
	Patient must have significant Fabry-related gastrointestinal symptoms despite the use of the recommended doses of appropriate pharmacological therapies.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: If hypertension is present in patients relying their eligibility on Fabry-related cardiac disease, the prescriber must treat it optimally for at least 6 months prior to submitting the first PBS authority application.
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).

	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Continuing treatment
	Clinical criteria:
	Patient must have received prior PBS-subsidised treatment with this drug for this condition
	AND
	Clinical criteria:
	Patient must have demonstrated clinical improvement or stabilisation of condition, the details of which must be kept with the patient's record
	AND
	Clinical criteria:
	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).

	Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Grandfather arrangement (transition from LSDP-funded Fabry disease therapy)
	Clinical criteria:
	Patient must have previously received treatment with enzyme replacement therapy for this condition funded under the Australian Government's Life Saving Drugs Program (LSDP) prior to [PBS listing date].
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease
	Prescribing Instructions: A patient may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a Grandfathered patient must qualify under the Continuing treatment criteria.
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).

	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Switching from PBS-subsidised ~~migalastat~~ *treatment*
	Clinical criteria:
	Patient must have received prior treatment with *any of the* PBS subsidised *treatments* ~~migalastat~~ for this condition
	AND
	Clinical criteria:
	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	AND
	Clinical criteria:
	Patient must not have failed to demonstrate clinical improvement or stabilisation of this condition with previous PBS-subsidised treatment with this drug.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.

	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).

MEDICINAL PRODUCT **medicinal product pack**	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
AGALSIDASE BETA					
agalsidase beta 5 mg injection, 1 vial	NEW HSD public	1	1	5	Fabrazyme
agalsidase beta 35 mg injection, 1 vial	NEW HSD private	1	1	5	
	Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)				
	Prescriber type: ☒ Medical Practitioners				
	Benefit type: ☒ Authority Required (FULL assessment) in writing via OPA/post/HPOS upload)				
	Authority type: ☒ Complex Authority Required (CAR)				
	Prescribing rule level:				
	Administrative Advice: No increase in the maximum number of repeats may be authorised.				
	Administrative Advice: Special Pricing Arrangements apply.				

**Same set of restrictions as presented above apply to agalsidase beta*

3.3 The PBAC had previously advised that Grandfathering arrangements for migalastat and pegunigalsidase alfa should not be time limited to provide for transition of patients treated under the LSDP (Paragraph 7.10, pegunigalsidase alfa PSD, July 2025 PBAC meeting). The PBAC was requested to re-consider this advice in light of transition of agalsidase alfa and beta from the LSDP to the PBS (the two remaining ERTs under the LSDP). The PBAC was also requested to consider a grandfather arrangement for agalsidase alfa and beta with a 12-month standard timeframe. If recommended, the PBAC was asked

if it was appropriate for the 12-month standard timeframe to flow-on to the grandfather arrangements of the other PBS-listed/PBAC recommended FD treatments.

3.4 Considering that agalsidase alfa and agalsidase beta are administered at a variable dose (i.e., a weight-based dosing regimen), the Secretariat proposed a maximum quantity of 1 and to include a Prescriber Instruction that instructs prescribers to request the appropriate quantity based on patient's weight consistent with the listing of pegunigalsidase alfa. The PBAC is asked to advise if this is appropriate.

3.5 As per the TGA Product Information (PI), agalsidase alfa is only recommended for patients who are 6.5 years and older and agalsidase beta is recommended for patients who are 8 years and older. The TGA PI for agalsidase beta states that the safety and efficacy in patients younger than 8 years of age have not been evaluated. The PBAC was therefore asked if it was appropriate for the listings of agalsidase alfa and agalsidase beta to be age agnostic consistent with the recommended restriction of pegunigalsidase alfa.

3.6 As per the LSDP guidelines, the Secretariat noted confirmation by renal biopsy is recommended for Fabry-related renal disease or a confirmation by myocardial biopsy is recommended for Fabry-related cardiac disease. These criteria were not included in the restrictions for migalastat and pegunigalsidase alfa. The PBAC was therefore asked to advise if confirmation by biopsy for Fabry-related renal disease or cardiac disease is required for the restrictions for agalsidase alfa and agalsidase beta.

3.7 In May 2024 the PBAC recommended an amendment to the cardiac restriction criterion for migalastat to include late gadolinium enhancement or low T1 changes on cardiac magnetic resonance imaging. Inclusion of this criterion was recommended for pegunigalsidase alfa and was hence included in the proposed restrictions for agalsidase alfa and beta (see also estimated PBS usage and financial implications).

3.8 Consistent with the listing of pegunigalsidase alfa (but unlike that of migalastat), the proposed PBS restrictions for agalsidase alfa and agalsidase beta do not include the clinical criterion 'Patient must have an estimated glomerular filtration rate (eGFR) of at least 30 mL/min/1.73 m2', as the TGA PIs state that no dose adjustment is needed in patients with renal impairment.

3.9 Consistent with the listing of pegunigalsidase alfa and migalastat, the proposed PBS restrictions for agalsidase alfa and agalsidase beta include the clinical criterion 'The treatment must be the sole PBS-subsidised therapy for this condition' to prevent the concomitant use of FD therapies.

3.10 Early Supply Rule should not apply to agalsidase alfa and agalsidase beta as it does not apply to prescriptions generated by hospitals (i.e. under HSD). The PBAC previously recommended that the Early Supply Rule should not apply to pegunigalsidase alfa (paragraph 7.18, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

3.11 If recommended for listing, the PBAC must make a recommendation as to whether, under Section 101 (3BA) of the National Health Act 1953, agalsidase alfa, agalsidase beta and pegunigalsidase alfa should be treated as interchangeable on an individual patient basis. The PBAC previously recommended that pegunigalsidase alfa should not be treated as interchangeable with any other drugs (Paragraph 7.27, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

3.12 As part of the evaluation, the Sponsors were requested to provide completed Australian Digital Health Agency (ADHA) Australian Medicines Terminology (AMT) mapping forms for agalsidase alfa and agalsidase beta in their pre-PBAC responses to the Department.

For more detail on PBAC's view, see section 7 PBAC outcome.

4 Comparator

4.1 Pegunigalsidase alfa is nominated as the comparator.

4.2 The referral of agalsidase alfa and beta is based upon PBAC consideration of pegunigalsidase alfa at its July 2025 meeting. The PBAC considered agalsidase alfa and beta would have been the appropriate comparators for pegunigalsidase alfa, however as they were not PBS listed, migalastat being PBS listed was the appropriate comparator (Paragraph 7.7, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

4.3 As part of its recommendation for the listing of pegunigalsidase alfa the PBAC considered the claim that pegunigalsidase alfa has non-inferior efficacy compared to agalsidase beta was adequately supported. The PBAC considered there was a lack of clinical evidence to support the claim of non-inferior efficacy compared to agalsidase alfa, however it was reasonable to conclude that agalsidase alfa has similar efficacy to agalsidase beta, and therefore pegunigalsidase alfa (Paragraph 7.4, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

4.4 The PBAC considered the claim of non-inferior safety compared to agalsidase alfa, agalsidase beta to be reasonable (Paragraph 7.6, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

For more detail on PBAC's view, see section 7 PBAC outcome.

5 Consideration of the evidence

Sponsor comments

Takeda Pharmaceuticals Australia Pty Ltd Response

Sanofi Australia and New Zealand

Consumer input

5.1 The PBAC noted and welcomed the input from individuals (21), health care professionals (1) and organisations (4) via the Consumer Comments facility on the PBS website.

5.2 The comments from individuals noted the current benefits of agalsidase alfa and beta treatment through the LSDP and concerns over the restrictive nature of the LSDP eligibility criteria when compared to PBS recommended alternatives.

5.3 Comments regarding agalsidase alfa and beta for Fabry disease were received from the following organisations:

- Australasian College of Dermatologists
- Fabry Australia
- Fabry Australia – Medical Advisory Committee
- Australian Pompe Association

5.4 The PBAC noted that the advice from health care professionals, individuals and organisations was supportive of the evidence provided in the submission and for a broader listing criteria as recommended for pegunigalsidase alfa. The PBAC further noted concerns around any future provision of sponsor provided home infusion services.

6 Pricing considerations

Drug cost/patient/year:

6.1 Agalsidase alfa and beta were considered on a cost minimisation basis to pegunigalsidase alfa. At the time of PBAC consideration, pegunigalsidase alfa was not yet PBS-listed but progressing through the listing process.

6.2 At its July 2025 meeting the PBAC advised that the equi-effective dose of pegunigalsidase alfa was 78.9 mg every two weeks to migalastat 123 mg every other day (Paragraph 7.9, pegunigalsidase alfa PSD, July 2025 PBAC meeting). This was based on the average weight

of participants in the BALANCE trial of 78.9 kg and the recommended dose in the pegunigalsidase alfa Product Information of 1 mg/kg. The PBAC further advised at its July 2025 meeting that the annual cost of pegunigalsidase alfa should not exceed the cost of 12 packs of migalastat per year (Paragraph 7.8, pegunigalsidase alfa PSD, July 2025 PBAC meeting).

6.3 The proposed equi-effective doses for agalsidase alfa and beta were based on the TGA Product Information:

- Agalsidase alfa: 0.2 mg/kg every two weeks
- Agalsidase beta: 1 mg/kg every two weeks
- pegunigalsidase alfa 1 mg/kg every two weeks
- migalastat 123 mg every other day

Estimated PBS usage & financial implications

6.4 The PBAC noted that the financial implications for the listing of pegunigalsidase alfa were uncertain (Paragraph 7.13, pegunigalsidase alfa PSD, July 2025 PBAC meeting). This uncertainty included uptake rates of patients switching from LSDP listed treatments to pegunigalsidase alfa.

6.5 The PBAC had considered it would be easier for clinicians to prescribe pegunigalsidase alfa under the PBS compared to treatments on the LSDP (Paragraph 7.13, pegunigalsidase alfa PSD, July 2025 PBAC meeting). However, PBAC consideration of a PBS listing of agalsidase alfa and beta may influence prescriber behaviour in relation to the option to remain on current therapies, should this outcome be available.

6.6 Listing of pegunigalsidase alfa had yet to eventuate at the time of PBAC consideration. Therefore, the presented utilisation and financial implications presented the current situation and transition of all patients to the PBS at the same cost as pegunigalsidase alfa.

6.7 Listing of agalsidase alfa and beta is proposed under the same conditions the PBAC recommended for the listing of pegunigalsidase alfa. At its July 2025 meeting the PBAC advised that the clinical criterion relating to Fabry-related cardiac disease for pegunigalsidase alfa be aligned with migalastat's listing, including diagnosis of FD by late gadolinium enhancement or a low T1 on cardiac MRI (Paragraph 7.10, pegunigalsidase alfa PSD, July 2025 PBAC meeting). The PBAC noted this clinical criterion is broader than the current LSDP criteria for agalsidase alfa and beta.

6.8 The PBAC recommended risk share arrangements (RSA) for both migalastat and pegunigalsidase alfa.

6.9 The CIC section presents current LSDP utilisation data and estimated financial impact based on a cost-minimisation to pegunigalsidase alfa.

Committee-In-Confidence information

6.10 [redacted]

[redacted]

6.11 [redacted]

6.12 [redacted]

[redacted]

6.13 [redacted]

6.14 [redacted]

6.15 [redacted]

6.16 [redacted]

For more detail on PBAC's view, see section 7 PBAC outcome.

Quality Use of Medicines

6.17 The sponsor of pegunigalsidase alfa will coordinate a home infusion program (through a third-party provider) and provide accredited nurses to deliver infusions (Paragraph 6.94, pegunigalsidase alfa PSD, July 2025 PBAC meeting)

6.18 [redacted]

End Committee-In-Confidence information

7 PBAC Outcome

7.1 The PBAC recommended the listing of agalsidase alfa and agalsidase beta for the treatment of Fabry Disease (FD) under the same conditions as recommended for pegunigalsidase alfa; Authority Required (Written) listing for initial treatment and an Authority Required (telephone/electronic) listing for continuing treatment, on the basis that it should be available only under the Section 100 Highly Specialised Drugs Program (Public and Private)). At the time of PBAC consideration agalsidase alfa and beta were funded on the Life Saving Drugs Program (LSDP).

7.2 The PBAC noted the broad support from clinicians, peak bodies and consumers for the inclusion of agalsidase alfa and beta on the PBS, under the same conditions as recommended for the PBS listing of pegunigalsidase alfa.

7.3 The PBAC noted consumer concerns around the provision of sponsor funded home infusions services for agalsidase alfa and beta, but that such arrangements were commercial matters for the medicine sponsors. The PBAC further noted that the sponsor of pegunigalsidase alfa would be supporting home infusion services.

7.4 The PBAC considered pegunigalsidase alfa was the appropriate comparator in respect of clinical efficacy, safety and cost-effectiveness. The PBAC considered that migalastat was also an appropriate comparator, noting that pegunigalsidase alfa was recommended on a cost-minimisation basis with migalastat.

7.5 The PBAC's recommendation for listing was based on cost minimisation of agalsidase alfa and beta to pegunigalsidase alfa, in the event that it is listed on the PBS. The PBAC advised the equi-effective doses could be derived with reference to the relevant TGA Product Information as follows:

- agalsidase alfa 0.2 mg/kg every two weeks
- agalsidase beta 1 mg/kg every two weeks
- pegunigalsidase alfa 1 mg/kg every two weeks
- migalastat 123 mg every other day

7.6 The PBAC recalled that at its July 2025 meeting it had previously considered the claim of non-inferior comparative effectiveness and non-inferior comparative safety of pegunigalsidase alfa compared to agalsidase alfa and beta was reasonable.

7.7 The PBAC advised that the restrictions for agalsidase alfa and agalsidase beta should align with those recommended for pegunigalsidase alfa, including the following:

- A Grandfather (Authority Required (Written)) listing to allow patients who are currently treated with LSDP-funded agalsidase alfa or beta to transition to the PBS-subsidised treatment.
- Inclusion of a 12-month standard timeframe for the grandfather restriction when the last enzyme replacement therapy transitions from the LSDP.
- A switching treatment phase to allow switching from any of the PBS-listed treatments for FD to agalsidase alfa or beta.

- The maximum quantity of 1, with prescribers able to request the number of vials required, based on the patient's weight, to allow for 4 weeks of treatment. Each treatment phase would allow up to 5 repeats.
- The listing be age-agnostic.
- Confirmation by biopsy for Fabry-related renal disease or cardiac disease not required for the restrictions.
- Inclusion of the clinical criterion relating to Fabry-related cardiac disease of late gadolinium enhancement or low T1 on cardiac MRI.
- Exclusion of the clinical criterion 'Patient must have an estimated glomerular filtration rate (eGFR) of at least 30 mL/min/1.73 m^2', as the TGA PIs state that no dose adjustment is needed in patients with renal impairment.
- Inclusion of the clinical criterion 'The treatment must be the sole PBS-subsidised therapy for this condition' to prevent the concomitant use of FD therapies.
- Inclusion of an administrative advice stipulating that the PBS-subsidised treatments for Fabry disease are agalsidase alfa, algalsidase beta, migalastat and pegunigalsidase alfa.

7.8 The PBAC recommended the following flow-on changes to the recommended listing of pegunigalsidase alfa and the PBS listing of migalastat:

- Amendment of the restriction for switching to provide for patients to switch between migalastat, pegunigalsidase alfa, agalsidase alfa and agalsidase beta.
- Inclusion of the 12-month standard timeframe to the grandfather restriction of the PBS listing of migalastat and the recommended listing of pegunigalsidase alfa when the last enzyme replacement therapy transition from the LSDP, noting patients will not be recommencing treatment under the LSDP once an enzyme replacement therapy is listed on the PBS.
- Inclusion of an administrative advice stipulating that the PBS-subsidised treatments for Fabry disease are agalsidase alfa, algalsidase beta, migalastat and pegunigalsidase alfa

7.9 The PBAC advised that agalsidase alfa and agalsidase beta are suitable for prescribing by medical practitioners only, specifically physicians with expertise in the management of Fabry disease, and are not suitable for prescribing by nurse practitioners.

7.10 The PBAC noted that a proportion of LSDP Fabry disease patients were expected to transition to pegunigalsidase alfa (July 2025 PBAC Minutes, paragraph 7.13). The PBAC

considered that the financial estimates for agalsidase alfa and agalsidase beta should be based on the remaining LSDP patient population transitioning to the PBS. The PBAC noted that the inclusion of agalsidase alfa and beta on the PBS would be a net save to the Government from the funding of these medicines under the LSDP.

7.11 The PBAC advised that agalsidase alfa and agalsidase beta should join the risk sharing arrangement (RSA) for pegunigalsidase alfa, noting that each of agalsidase alfa, agalsidase beta and pegunigalsidase alfa are enzyme replacement therapies for the treatment of FD. The PBAC considered it would be appropriate for the caps to be increased by the incremental estimated PBS expenditure (as outlined in paragraph 7.10).

7.12 The PBAC noted it had previously requested a review of the utilisation of medicines for FD on the PBS be undertaken following two years of listing on the PBS of pegunigalsidase alfa as part of its recommendation for the PBS listing of pegunigalsidase alfa.

7.13 The PBAC recommended that agalsidase alfa and agalsidase beta should be treated as interchangeable with pegunigalsidase alfa.

7.14 The PBAC recommended that the Early Supply Rule should not apply.

7.15 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because agalsidase alfa and agalsidase beta are not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over pegunigalsidase alfa, or not expected to address a high and urgent unmet clinical need given the presence of an alternative therapy, the criteria prescribed by the National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022 for Pricing Pathway A were not met.

7.16 The PBAC noted that this submission is not eligible for an Independent Review because it received a positive recommendation.

Outcome:
Recommended

7 Recommended listing

7.1 Add new item:

Agalsidase alfa

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
AGALSIDASE ALFA					
agalsidase alfa 3.5 mg/3.5 mL injection, 3.5 mL vial	NEW HSD public NEW HSD private	1	1	5	Replagal
	Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)				
	Prescriber type: ☒ Medical Practitioners				
	Benefit type: ☒ Authority Required (FULL assessment) in writing via OPA/post/HPOS upload)				
	Authority type: ☒ Complex Authority Required (CAR)				
	Prescribing rule level:				
	Administrative Advice: PBS-subsidised treatments for Fabry disease are agalsidase alfa, agalsidase beta, migalastat and pegunigalsidase alfa.				
	Administrative Advice: No increase in the maximum number of repeats may be authorised.				
	Administrative Advice: Special Pricing Arrangements apply.				
Restriction Summary / Treatment of Concept					
	Indication: Fabry disease				
	Treatment Phase: Initial treatment				
	Clinical criteria:				
	Patient 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				
	Clinical criteria:				
	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 antiseizure medications for peripheral neuropathy; OR
	Patient must have significant Fabry-related gastrointestinal symptoms despite the use of the recommended doses of appropriate pharmacological therapies.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: If hypertension is present in patients relying their eligibility on Fabry-related cardiac disease, the prescriber must treat it optimally for at least 6 months prior to submitting the first PBS authority application.
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001

Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Continuing treatment
	Clinical criteria:
	Patient must have received prior PBS-subsidised treatment with this drug for this condition
	AND
	Clinical criteria:
	Patient must have demonstrated clinical improvement or stabilisation of condition, the details of which must be kept with the patient's record
	AND
	Clinical criteria:
	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Grandfather arrangement (transition from LSDP-funded Fabry disease therapy)
	Clinical criteria:
	Patient must have previously received treatment with enzyme replacement therapy for this condition funded under the Australian Government's Life Saving Drugs Program (LSDP) prior to [PBS listing date].
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease

	Prescribing Instructions: A patient may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a Grandfathered patient must qualify under the Continuing treatment criteria.
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
	Administrative Advice: This grandfather restriction will cease to operate from 12 months after [the date of the PBS listing of the last ERT transitioning from the LSDP (agalsidase alfa or agalsidase beta)]
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Switching from PBS-subsidised treatment
	Clinical criteria:
	Patient must have received prior treatment with any of the PBS subsidised treatments for this condition
	AND
	Clinical criteria:

	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	AND
	Clinical criteria:
	Patient must not have failed to demonstrate clinical improvement or stabilisation of this condition with previous PBS-subsidised treatment with this drug.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).

Agalsidase beta

MEDICINAL PRODUCT **medicinal product pack**	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
AGALSIDASE BETA					
agalsidase beta 5 mg injection, 1 vial	NEW HSD public	1	1	5	Fabrazyme
agalsidase beta 35 mg injection, 1 vial	NEW HSD private	1	1	5	
	Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)				
	Prescriber type: ☒ Medical Practitioners				
	Benefit type: ☒ Authority Required (FULL assessment) in writing via OPA/post/HPOS upload)				

	Authority type: ☒ Complex Authority Required (CAR)
	Prescribing rule level:
	Administrative Advice: PBS-subsidised treatments for Fabry disease are agalsidase alfa, agalsidase beta, migalastat and pegunigalsidase alfa.
	Administrative Advice: No increase in the maximum number of repeats may be authorised.
	Administrative Advice: Special Pricing Arrangements apply.
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Initial treatment
	Clinical criteria:
	Patient 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
	Clinical criteria:
	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 antiseizure medications for peripheral neuropathy; OR
	Patient must have significant Fabry-related gastrointestinal symptoms despite the use of the recommended doses of appropriate pharmacological therapies.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: If hypertension is present in patients relying their eligibility on Fabry-related cardiac disease, the prescriber must treat it optimally for at least 6 months prior to submitting the first PBS authority application.

	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Continuing treatment
	Clinical criteria:
	Patient must have received prior PBS-subsidised treatment with this drug for this condition
	AND
	Clinical criteria:
	Patient must have demonstrated clinical improvement or stabilisation of condition, the details of which must be kept with the patient's record
	AND
	Clinical criteria:

	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug.
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Grandfather arrangement (transition from LSDP-funded Fabry disease therapy)
	Clinical criteria:
	Patient must have previously received treatment with enzyme replacement therapy for this condition funded under the Australian Government's Life Saving Drugs Program (LSDP) prior to [PBS listing date].
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease
	Prescribing Instructions: A patient may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a Grandfathered patient must qualify under the Continuing treatment criteria.
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include and must include: (1) details of the proposed prescription(s); and (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).

	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
	Administrative Advice: This grandfather restriction will cease to operate from 12 months after [the date of the PBS listing of the last ERT transitioning from the LSDP (agalsidase alfa or agalsidase beta)]
Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Switching from PBS-subsidised treatment
	Clinical criteria:
	Patient must have received prior treatment with any of the PBS subsidised treatments for this condition
	AND
	Clinical criteria:
	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	AND
	Clinical criteria:
	Patient must not have failed to demonstrate clinical improvement or stabilisation of this condition with previous PBS-subsidised treatment with this drug.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease

	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).

Flow-on changes

7.2 Addition of the following AA at the PR level to PBS-listed migalastat (PBS item: 14573B) and the recommended listing of pegunigalsidase alfa when listed for the treatment of FD.

	Administrative Advice: PBS-subsidised treatments for Fabry disease are agalsidase alfa, agalsidase beta, migalastat and pegunigalsidase alfa.

7.3 Only add the following AA when the last ERT transition from LSDP: Addition of the following AA in the 'Treatment Phase: Grandfather arrangement (transition from LSDP-funded Fabry disease therapy) to PBS-listed migalastat (PBS item:14573B) and the recommended listing of pegunigalsidase alfa when listed for the treatment of FD.

	Administrative Advice: This grandfather restriction will cease to operate from 12 months after *[the date of the PBS listing of the last ERT transitioning from the LSDP (agalsidase alfa or agalsidase beta)]*

7.4 Add new 'switching treatment phase' to PBS-listed migalastat (PBS item: 14573B) and the recommended listing of pegunigalsidase alfa when listed to allow patients to switch between any of the PBS-subsidised treatments for FD.

Restriction Summary / Treatment of Concept	
	Indication: Fabry disease
	Treatment Phase: Switching from PBS-subsidised treatment
	Clinical criteria:
	Patient must have received prior treatment with any of the PBS-subsidised treatments for this condition

	AND
	Clinical criteria:
	Patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug
	AND
	Clinical criteria:
	The treatment must be the sole PBS-subsidised therapy for this condition.
	AND
	Clinical criteria:
	Patient must not have failed to demonstrate clinical improvement or stabilisation of this condition with previous PBS-subsidised treatment with this drug.
	Treatment criteria:
	Must be treated by a physician with expertise in the management of Fabry disease
	Prescribing Instructions: Confirmation of eligibility for treatment with diagnostic reports including the confirmed mutations must be documented in the patient's medical records.
	Prescribing Instructions: At the time of authority application, prescribers must request the appropriate number of vials, based on the weight of the patient, to provide sufficient drug for 4 weeks of treatment at the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI).
	Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).

These restrictions may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed.

8 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

9 Sponsor's Comment

The sponsor had no comment.