

Terms of Reference for the Review of Life Saving Drugs Program medicines for the Treatment of Fabry disease

Medicines under review:

- Agalsidase alfa (Replagal®); and
- Agalsidase beta (Fabrazyme®)

1. Review the prevalence of Fabry disease within Australia.
2. Review evidence for the management of Fabry disease and compare to the LSDP treatment guidelines, patient eligibility and testing requirements for the use of these medicines on the program (including the validity of the tests).
3. Review clinical effectiveness and safety of medicines and evaluate the evidence of comparative effectiveness of LSDP Fabry disease medicines. This will include analysis of LSDP patient data and international literature to provide evidence of life extension.
4. Review relevant patient based outcomes that are most important or clinically relevant to patients with Fabry disease.
5. Conduct an analysis of the value for money of LSDP Fabry disease medicines under the current funding arrangements.
6. Review the utilisation of LSDP Fabry disease medicines, including the way they are stored and dispensed, and evidence of patient compliance to treatment.
7. Investigate developing technologies that may impact future funded access.