

Terms of Reference for the Review of Life Saving Drugs Program alglucosidase alfa for the Treatment of Infantile-onset, Juvenile Late- onset or Adult Late-onset Pompe disease

Medicine under review:

- Alglucosidase alfa (Myozyme®)
1. Review the prevalence of Pompe disease within Australia, both split by type (infantile onset, juvenile late-onset, adult late-onset) and overall.
 2. Review evidence for the management of each type of Pompe 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 alglucosidase alfa in each of the treated populations. 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 each type of Pompe disease.
 5. Assess the value for money of alglucosidase alfa in each of the treated populations under the current funding arrangements by evaluating the benefit of the drug's treatment outcomes and cost.
 6. Review the utilisation of alglucosidase alfa in each of the treated populations, including storage, dispensing and evidence of patient compliance to treatment.
 7. Investigate developing technologies that may impact future funded access.