

Terms of Reference for the Review of Life Saving Drugs Program idursulfase for the Treatment of Mucopolysaccharidosis Type II (MPS II)

Medicine under review:

- Idursulfase (Elaprase[®])

1. Review the prevalence of Mucopolysaccharidosis Type II (MPS II) within Australia.
2. Review evidence for the management of MPS II 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. 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 MPS II.
5. Assess the value for money of idursulfase under the current funding arrangements by evaluating the benefit of the drug's treatment outcomes and cost.
6. Review the utilisation of idursulfase, including storage, dispensing and evidence of patient compliance to treatment
7. Investigate developing technologies that may impact future funded access.