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

Medicine under review:
• Laronidase (Aldurazyme®)

1. Review the prevalence of Mucopolysaccharidosis Type I (MPS I) within Australia.

2. Review evidence for the management of MPS I and compare to the LSDP treatment guidelines, patient eligibility and testing requirements for the use of this medicine 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 I.

5. Conduct an analysis of the value for money of LSDP laronidase under the current funding arrangements.

6. Review the utilisation of laronidase, including the way it is stored and dispensed, and evidence of patient compliance to treatment.

7. Investigate developing technologies that may impact future funded access.