Fact Sheet: Medicines for rare and ultra-rare diseases

Medicines Australia

The HTA Review recommends streamlining HTA of medicines for rare and ultra-rare diseases to speed up patient access to these medicines. The proposed restructuring of the Life-Saving Drugs Program (LSDP) to allow the PBAC to recommend listing of health technologies on the LSDP complements this recommendation.

The Review makes many recommendations that will reduce the time taken for Australians to gain access to new treatments for rare and ultra-rare diseases.

These include:

- Developing a statement of rationale for the LSDP, covering principles and eligibility criteria, including value-for-money considerations (Recommendation 14).
- Streamlining processes for the LSDP (Recommendation 4) and expanding the role of the PBAC beyond the PBS to allow it to recommend medicines for inclusion on the LSDP (Recommendation 6).
- Early identification of real-world data sources to support submissions that may require a managed access approach, including international collaboration on collection of patient-level data where possible for treatments for ultra-rare diseases or small patient populations (complemented by local usage data) (Recommendation 31).
- Updating guidance on the use of lower-level (non-RCT) data, including real-world data, developed in consultation with stakeholders (Recommendation 35).
- Development of additional methods for the use of surrogate endpoints, tumour-agnostic therapies, and genomic technologies and gene therapies (Recommendations 36 and 38).
- Proactively identifying and encouraging HTA submissions for reimbursement of therapies for conditions of high unmet clinical need (Recommendations 44-46).