Medicines Australia | Better health through research and innovation

HTA and rare diseases

October 2022

Access to life-changing therapies must be improved for the 8% of Australians living with a rare disease

- Australians currently wait longer for access to innovative therapies for rare diseases than patients in other OECD countries
- In some cases, Australian patients miss out entirely on rare disease treatments that are available overseas[1], where countries have processes specifically tailored for rare disease therapies
- Improving access to innovative therapies for Australians with rare diseases must be a priority for Australia's independent review of Health Technology Assessment (HTA) processes in 2022-23

Possible Policy Solutions

1. Provide a direct path to funding via the Life Saving Drugs Program (LSDP). The LSDP pays for specific essential medicines to treat patients with rare and life-threatening diseases. Current LSDP listing processes are lengthy and inefficient, as an innovative medicine must first be rejected by the Pharmaceutical Benefits Advisory Committee (PBAC) before being considered for funding on the LSDP.
2. Recognise severe disability and/or quality of life in the eligibility criteria for the LSDP. Funding decisions don't always take into account the full complexities of rare diseases and the value of innovative medicines that improve quality of life and address disability, as well as those that extend life.
3. Provide streamlined pathways for funding of rare diseases medicines. This may include:
    - Limited use of Incremental Cost Effectiveness Ratios (ICERs) in decision making for rare disease. Instead, use multi-criteria decision analysis that incorporates overall budgetary impact, health gains and the flow-on effect on society.
    - Greater acceptance of Real-World Evidence and registry data.
    - Consideration of caregiver burden for life long chronic rare diseases.

    Eligibility for this pathway should be aligned with the Therapeutic Goods Administration (TGA) definition of rare diseases.

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1 Jackson A, Geatches L, The McKell Institute 2021 Progress Update: Funding Rare Disease Therapies in Australia - Ensuring Equitable Access to Healthcare for all Australians, The McKell Institute, Australia, November 2021 https://mckellinstitute.org.au/wp-content/uploads/2022/02/McKell-Funding-Rare-Disease-Therapies-in-Aus-2021.pdf

Access issues faced by Australians living with rare diseases

Australians currently wait between two to four years longer than comparable countries to access rare disease medicines[1]. 'Orphan drugs', which target rare diseases, take longer to secure access, compared to other therapies in all countries except Germany and Scotland[2].

Japan, Germany, the UK and France have developed specific processes to overcome the challenges of patient access to orphan drugs. These include aspects such as supplementary processes, exclusion of cost-effectiveness analysis in HTA and higher or more flexible thresholds for funding.

HTA undertaken by the PBAC for reimbursement purposes doesn't always acknowledge the full complexity of rare diseases, and applies inappropriately high evidence thresholds. The recent review of NICE (England's HTA agency) Methods highlighted the need for different evidence standards to be applied to orphan drugs, and the need for greater acceptance of real world evidence. That is, evidence that is generated through the day-to-day use of the therapy and not in clinical trials. This reflects the challenges of conducting clinical trials in rare diseases with small patient populations.

LSDP eligibility criteria require a therapy to demonstrate that it is "life-saving", which excludes interventions that substantially improve quality of life. This can be especially important for patients with significant disability and the social benefits of these interventions.

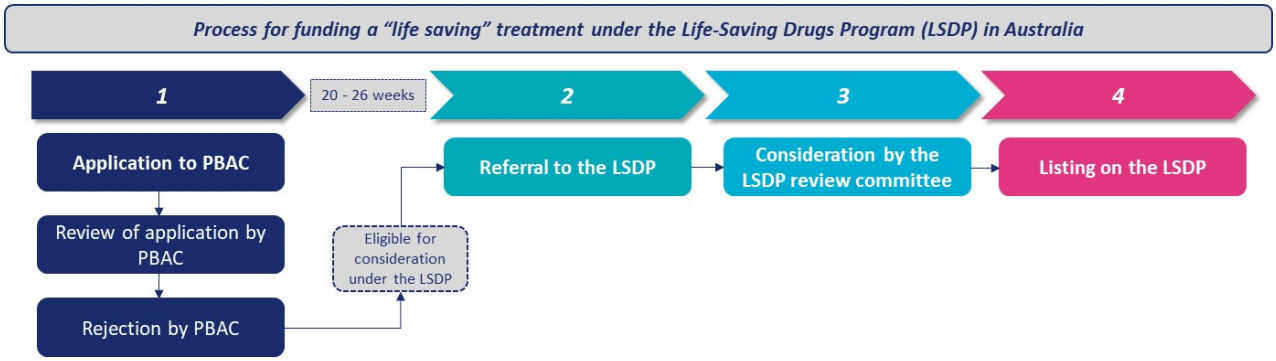
Rare disease definitions differ across agencies and programs, which can lead to inconsistencies across rare disease policies. The National Strategic Action Plan for Rare Diseases defines a rare disease as "one that affects less than five in 10,000 people", which aligns with international definitions and TGA criteria for 'orphan drugs'[3]. A much more restrictive definition is applied for eligibility for the LSDP, of fewer than one in 50,000 people.

Processes for funding therapies on the LSDP are long and inefficient. As detailed in Figure 1, A "life-saving" treatment for an ultra-rare disease therapy must first be 'rejected' by the PBAC before it can be considered for funding under the LSDP. LSDP Procedure Guidance recommends 20-26 weeks between a PBAC meeting and an LSDP recommendation to the Minister. These processes add a minimum 12-month delay to the reimbursement process.

2 Centre for Innovation in Regulatory Science (CIRS), CIRS RD Briefing 83 – HTA outcomes in Australia, Canada and Europe 2016-2020, CIRS, UK, September 2021
https://cirsci.org/wp-content/uploads/dlm_uploads/2021/08/CIRS-HTADock-briefing-RDB83_Final__280122.pdf

3 Australian Government Department of Health and Aged Care, National Strategic Action Plan for Rare Diseases, Australian Government, Australia, February 2020
https://www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases

Figure 1: Process for funding a "life-saving" treatment under the LSDP



Addressing the Australian patient access gap for rare disease

Approximately two million Australians are estimated to be living with a rare disease, around 80% of which are genetic[1]. Innovative therapies are being developed, globally, to target rare diseases.

The Strategic Agreement between the Australian Government and Medicines Australia outlines a shared commitment to "ensure access for Australian patients to the rapid advances in modern, and emerging, technologies, therapies and vaccines, and to address the complexities of enabling access for therapies to treat rare diseases".

The Strategic Agreement also makes a commitment to review HTA policy and methods, which is the first comprehensive review of Australian HTA in 30 years. This is an important opportunity to introduce bold reforms to address the Australian patient access gap for people living with rare diseases.

Feedback

Do you have any thoughts on the policy ideas in these papers? We'd love to hear your feedback! Please let us know at this email address: HTA-Reform@medicinesaustralia.com.au.