The Life Saving Drug Program: Australia's pathway to high-cost drugs

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policy brief

Australia's approach to expanding high-cost drug access

Australia developed the Life-Saving Drugs Program (LSDP) in 1995 to complement the Pharmaceutical Benefits Scheme (PBS), expanding access to high-cost drugs for rare diseases (DRD). This program, funded and administered by the Department of Health and Aged Care, permitted sponsors (often pharmaceutical companies) to apply for listing when their clinically effective DRD is rejected for PBS listing on the grounds of cost-effectiveness.[1] At the time of writing (2022), sixteen medicines were subsidised via the LSDP.[2]

The Pharmaceutical Benefits Scheme contains a list of medicines the Australian government subsidises to reduce the out-of-pocket costs that beneficiaries pay to access medicines. The Pharmaceutical Benefits Advisory Committee (PBAC), a government-appointed independent expert body, uses a set of criteria, including cost-effectiveness, to evaluate whether a medicine should be included in the PBS.[3] Although cost-effectiveness analysis is a legislative requirement, the PBAC does not use a defined Incremental Cost-effectiveness Ratio (ICER) threshold. Nevertheless, past studies have shown that medicines with lower cost-effectiveness ratios have a higher chance of listing.[4]

The consideration of cost-effectiveness is crucial for budgetary control, but it poses a challenge when assessing DRDs. This is because DRDs have a weaker evidence base for their effectiveness and higher prices due to higher research costs and fewer competitors in smaller-sized markets.[5]

Table 1: Drugs reimbursed through LSDP in 2022 (adapted from the LSDP website)

Medicine(s)	Condition
Agalsidase alfa (Replagal®) Agalsidase beta (Fabrazyme®) Migalastat (Galafold®)	Fabry disease
Imiglucerase (Cerezyme®) Velaglucerase (VPRIV®) Taliglucerase (Elelyso®)	Gaucher disease (type 1)
Nitisinone (Orfadin® and Nityr™)	Hereditary tyrosinaemia type 1 (HT1)
Cerliponase alfa (Brineura®)	Late-infantile onset Batten disease (CLN2)

Medicine(s)	Condition
Laronidase (Aldurazyme®)	Mucopolysaccharidosis type I (MPS I)
Idursulfase (Elaprase®)	Mucopolysaccharidosis type II (MPS II)
Elosulfase alfa (Vimizim®)	Mucopolysaccharidosis type IVA (MPSIVA)
Galsulfase (Naglazyme®)	Mucopolysaccharidosis type VI (MPS VI)
Asfotase alfa (Strensiq®)	Perinatal- and infantile-onset hypophosphatasia (HPP)

The Life Saving Drug Program at a glance

After a medicine is rejected by PBAC, the sponsor applies for an LSDP listing with the required information. The LSDP Expert Panel then reviews the application, the LSDP secretariat's assessment of the application, additional stakeholder input from the public, presentations made to the panel, and materials from the PBAC's consideration to advise the Chief Medical Officer.[6] Within two to six weeks, the Chief Medical Officer makes a recommendation on whether the medicine should be funded through the LSDP, pending approval from the Minister for Health.[4] *See Figure 1 for the LSDP process.*

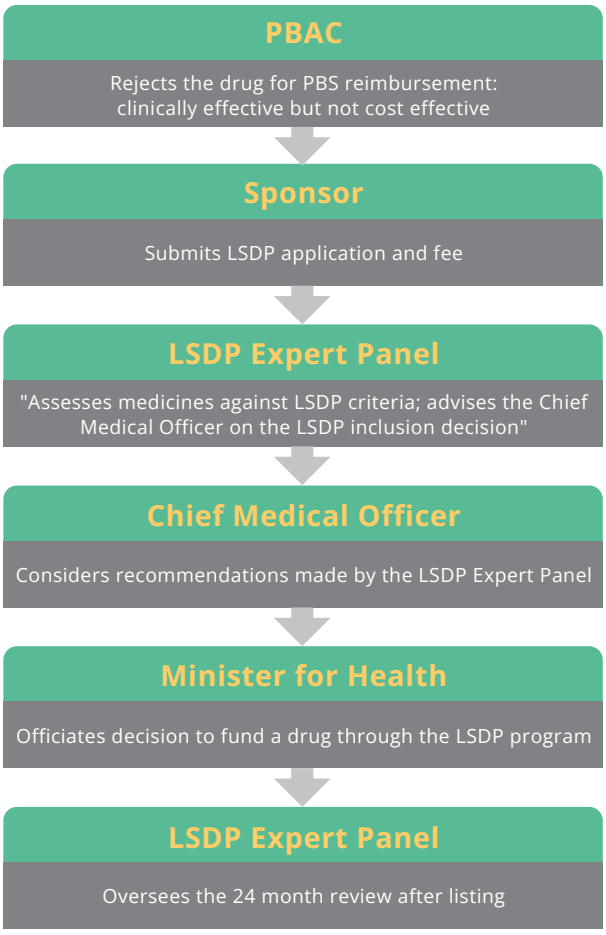

Figure 1: A simplified flowchart of the LSDP governance structure adapted from the procedure guidance.[4]

This section describes a simplified overview of the LSDP decision process. In practice, there is communication between the sponsor and the LSDP Expert Panel, where the sponsor supplies additional evidence upon request to support the decision-making. During the process, stakeholders such as patients, their caregivers, and physicians are welcome to directly provide written input to the LSDP Secretariat to be considered by the Expert Panel.[1] *The main stakeholders are depicted in Figure [2].*

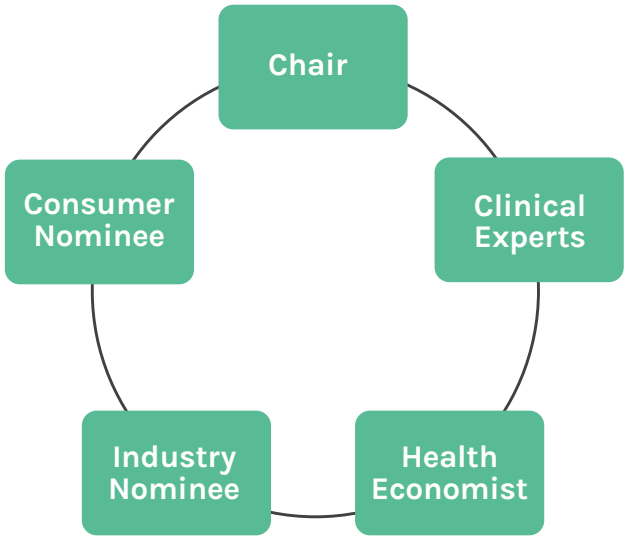

Figure 2: Key people on the LSDP Expert Panel [6]

Reimbursement Process

For a medicine to be recommended for LSDP listing, the LSDP Expert Panel assesses the application to ensure it meets the ten LSDP criteria. The requirements include disease rarity, defined as less than 1 in 50,000 people, as the LSDP is intended to supplement the PBS to expand access to DRD.[1] In addition, there must be evidence that the medicine can extend life or reduce disability in someone who would otherwise have a significant life reduction or have a significant disability due to the disease.[1] Although demonstrating the medicine is cost-effective is not required for listing, the sponsors are still needed to supply medicine prices in comparable overseas markets to provide the context of medicine pricing.[1] *See Table 2 for the complete list of criteria.*

Table 2: Decision-making criteria for LSDP drugs (adapted from procedure guidelines)

	Criterion	Notes
A1	The drug is a proven therapy for a rare but clinically definable disease	• ≤1 per 50,000 • High lifelong cost burden
A2	The disease is identifiable with reasonable diagnostic precision	
A3	Evidence of significant reduction in age-specific life expectancy due to the disease	• Data for disease progression without treatment • Life extension can be represented by disability reduction
A4	Evidence of significant life extension due to the drug	• Or significant disability reduction • Surrogate outcomes data is acceptable if there is no survival data
A5	The drug is clinically effective but rejected for PBS listing due to the lack of cost-effectiveness	
A6	No lifesaving alternatives on the PBS listing or available through public hospitals	
A7	No suitable and cost-effective non-drug therapy	Such as surgery or radiotherapy
A8	The cost of the drug is required per year is an unreasonable financial burden for the patient	
B1	The proposed confidential price of the drug compared with effective price in comparable oversea markets	
B2	The proposed cost of the drug compared with the cost of comparable drugs already funded through the LSDP	

It is important to note that despite the criteria that exist, they, by design, allow for flexibility, subject to the LSDP Expert Panel's discretion.

Price negotiation begins once the sponsor is notified that the Chief Medical Officer intends to advise the Minister for Health to fund the medicine through LSDP. Although details of the pricing arrangement are strictly confidential between the sponsor and Australia, the procedural guidance for LSDP listing has referenced using outcome-based risk-sharing agreements as a pathway to reimbursement.[1]

An outcome-based risk-sharing agreement can be used to determine the future price of a medicine. This type of agreement permits the medicine to be funded with the condition that further data on disease stability and improvement must be collected to evaluate the appropriateness of the price. The price of a medicine is then reduced if new evidence suggests it is less effective than previously assumed.

However, due to the disease rarity, data collected from the small sample size often lack statistical significance, posing a challenge to their implementation.

The LSDP only funds medicine costs but may also cover the cost of importation and transportation to some extent when the manufacturer directly delivers the medicine to the place of administration.

In addition to using risk-sharing to manage medicine prices, LSDP also adopted a price reduction policy to control medicine prices similar to that of PBS.[4] For example, the medicine price was to be reduced by 5% on the 5th anniversary, another 5% on the 10th anniversary, and finally by 26.1% on the 15th anniversary of listing.[7] However, this policy has been discontinued for LSDP since June 2022.[8]

Implementation

Before funding the medicine, the LSDP Secretariat must finalise the treatment guideline based on the Expert Panel advice and by working with the sponsor and clinical experts.[1] This will include directions for initiation and continuing the treatment. Once the medicine is approved for funding, a patient must meet the eligibility criteria to access the medicine. This includes satisfying treatment criteria and consenting to data collection for medicine evaluation. In addition, the patient must show clinical improvement or at least stabilisation of the condition for continued access to the medicine.

Patient access to LSDP medicine is carefully managed. The treating physician must apply to the LSDP to initiate access to the medicine and nominate a dispensing pharmacy.[9] The LSDP medicine will be delivered to the pharmacy in the quantity of a one-month supply only at a time, ordered by the LSDP directly, due to the high-cost nature of these medicines.[9] Differing from PBS medicines, patients do not co-pay to access LSDP medicines.[10]

Monitoring and Evaluation (M&E)

To ensure the use and effectiveness of the medicine meet the expectations at the time of listing, medicines on the LSDP are reviewed for their usage, clinical benefits, and financial cost 24 months after listing.[1] Patient-level data is collected and submitted by the treating physician to the Department of Health and Aged Care following their website instructions annually to understand the real-world use.[1,11] The scope of the review is drafted based on issues identified by PBAC and LSDP Expert Panel when the medicine was considered for listing. In addition to patient-level data collected by the Department, sponsors can also submit additional data, including international evidence, to support the review.[1]

Upon completion of the review, the recommendations are made to the Minister, which may include changing the eligibility criteria or treatment guidelines, amendments to risk-sharing arrangements or the scope of data collection, referral to PBAC for PBS listing considerations, or the removal of such medicine from the LSDP listing.[6]

Lessons Learned

- Establishing a new reimbursement programme such as the LSDP for clinically effective but high-cost medicines can facilitate the decision-making process by easing the criteria for cost-effectiveness when the medicine is lifesaving (or disability-reducing), and there are no alternative treatment options.
- Payers can control prescribing volumes and expenditures by requiring approval for individual-patient use from the funding authority *before* the medicine is dispensed and granting continued medicine access conditional on demonstrated improvement or stabilization of the patient's condition.
- Risk-sharing agreements may be explored to facilitate patient access to lifesaving medicines with a higher level of uncertainty in clinical benefit and minimise the payer's financial risk, while being mindful of implementation barriers such as higher transaction and administrative costs.[12]
- A two-tier evaluation system (PBAC evaluation followed by LSDP expert panel evaluation), while appearing time-consuming, may be more efficient as a baseline evaluation has already been performed, i.e., clinical data has already been assessed by PBAC, and the LSDP expert panel does not repeat the process completely from the beginning.

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