Life Saving Drugs Program Expert Panel Submission to the Health Technology Assessment Policy and Methods Review Consultation 2 Options Paper

February 2024

The Life Saving Drugs Program (LSDP) Expert Panel (the Expert Panel) was established on 1 July 2018 following a decision of the Australian Government based on the recommendations arising from the 2014 Post-market Review of the LSDP. The Expert Panel has provided for a more transparent and rigorous assessment of medicines seeking reimbursement through the LSDP alongside the ongoing review of data collection and consideration of medicines' continued eligibility for inclusion in the program. The expertise of the Expert Panel is reflected in its membership which spans clinical, health economic and consumer representation.

The Expert Panel's role is not limited to assessing new medicines for listing on the program. It also undertakes reviews of existing LSDP medicines; considers changes to listing arrangements; reassesses the ongoing eligibility of medicines listed on the LSDP as required; provides advice on matters of policy where requested by Government; and performs other functions consistent with its terms of reference.

The Expert Panel welcomes approaches that would streamline the availability of clinically effective medicines to Australians who need them. However, the Expert Panel notes that, although the assessment process is a contributor to the timeframe for access to novel medicines, it is not the only contributor (as set out in the Health Technology Assessment Policy and Methods Review (HTA Review) technical paper on Australian market authorisation, funding and assessment pathways and timelines). An important factor is the timing of a sponsor's decision to apply for product registration in Australia, and then subsequently to seek public subsidy. The Expert Panel is aware that Government has a range of policies in place intended to encourage the submission of clinically effective medicines for potential marketing in Australia, and that Government cannot compel medicine sponsors to seek listing in Australia, recognising such decisions are ultimately based on commercial considerations. The Expert Panel would support further changes, whether through actions arising from the HTA Review or otherwise, that would provide greater transparency on the current listing process. This transparency should extend to providing greater detail of the progress of consideration of LSDP medicines for subsidy, in line with the level of detail on the Medicines Status Website for Pharmaceutical Benefits Scheme (PBS) medicines.

The Expert Panel has considered the proposals relating to the future operations of the LSDP in the HTA Review Consultation 2 Options Paper (the Consultation Paper). The Expert Panel notes its support for the intent of the proposals and provides the following additional comments.

1. **Develop and publish a statement of rationale for the LSDP outlining principles underpinning the program, and the eligibility criteria, including the value-for-money consideration by reference to the overarching recommendations of the LSDP Review Expert Panel recommendation.**

The Expert Panel endorses the development and publication of a statement of rationale for the LSDP.

The Expert Panel believes that this statement should articulate the rationale for funding medicines for ultra-rare conditions at prices not considered cost-effective by the Pharmaceutical Benefits Advisory Committee (PBAC). For example, the statement might recognise that high research and development costs for new therapies, coupled with the small patient populations affected by ultra-rare conditions, could act as a barrier to developing effective medicines for these conditions or

bringing them to a relatively small market. It might also note that many ultra-rare conditions disproportionately affect young children, leading to profound impacts on their primary carers and families, and that the LSDP reflects the compassionate views of Australians towards people facing extraordinary challenges.

The current program parameters, with the requirement that medicines are not cost-effective, creates challenges for the Expert Panel in ensuring that the prices of these non-cost-effective medicines are appropriate and within reasonable limits. The statement of rationale needs to provide greater detail about how LSDP pricing and eligibility policies can support appropriate pricing for these very high-cost therapies. This may require the introduction of additional pricing and eligibility parameters. The Expert Panel notes these considerations will be a matter for Government.

The Expert Panel recognises the need to provide commercial incentives to sponsors to supply medicines for ultra-rare conditions in the Australian market through non-cost-effective prices. However, this must be balanced with policies that will ensure patient access and the program's long-term sustainability while still encouraging investment into newer and more clinically effective medicines. From a practical point of view, the Expert Panel believes greater transparency on pricing expectations would reduce the negotiation times between the Department of Health and Aged Care and the sponsor and would facilitate faster patient access to medicines for ultra-rare conditions.

2. PBAC to become the sole HTA committee for drugs for ultra-rare diseases to eliminate double handling. The expertise on the LSDP expert panel will inform and support decisions regarding therapies for ultra-rare diseases.

The Expert Panel supports, in principle, the concept and practicality of having the PBAC as the sole HTA committee for all medicines under consideration for listing on both the LSDP and PBS. The Expert Panel emphasises the importance of understanding the details of how these changes would be implemented, including consequential changes to the operations of the LSDP. This detail would include, for example, the application and assessment processes for LSDP listing, noting that the LSDP has separate eligibility criteria that may not be explicitly addressed in a standard application for PBS listing.

There should also be consideration of when and how in the assessment process additional ultra-rare disease expertise would be sought, noting the points in the paragraph above. The Expert Panel notes that, while the Consultation Paper proposes retaining the expertise on the Expert Panel to inform and support decisions, as an entity, it may become superfluous to the assessment and listing process. This may be considered a pragmatic outcome of potential reforms. However, the Expert Panel considers that the ultra-rare disease expertise currently contained in the Expert Panel should not be lost even if the committee itself is no longer required, and this expertise should be available to inform PBAC consideration of ultra-rare disease therapies.

The Expert Panel notes that the Consultation Paper limits its proposals to assessment processes, rather than any changes to the LSDP structure which are beyond its scope. However, the Expert Panel's view is that if there is a transition to the PBAC providing the sole assessment of LSDP medicines, there should also be consideration of whether this should be reflected in changes to program administration. The Expert Panel's view is that there would be benefits in including non-cost-effective life-saving medicines for ultra-rare conditions in the PBS as a section 100 program or equivalent, rather than continuing the current administrative separation of medicines for ultra-rare diseases from PBS arrangements. Such benefits would go beyond HTA processes, but are

expected to include reducing burden on treating physicians and patients and providing greater certainty for patients about continued access to medicines.

Equally, the Expert Panel notes the broad scope of the potential outcomes from the HTA Review and the complexity of implementation. The Expert Panel supports the retention of the LSDP as a standalone program where such a transition could be achieved in the short-term in view of the identified benefits, and notes that this would not preclude the program's future inclusion in the PBS.

The Expert Panel acknowledges that the implementation details of any reforms will be a matter for Government, but notes the importance of considering these practicalities at an early stage. Some matters relating to implementation may be resolved or clarified in part through the development of a statement of rationale for the LSDP as proposed by the Reference Committee.

3. PBAC advises the Minister on key requirements to enable listing on the LSDP based on a comparative assessment of effectiveness and cost.

The Expert Panel supports that the PBAC should be the authority to advise the Minister in matters pertaining to listings to the LSDP, in line with its proposed role as the sole HTA committee for consideration of listings for both the LSDP and PBS.

As noted above, the Expert Panel's current role goes beyond the assessment of potential new listings against the LSDP eligibility criteria. For existing medicines, the Expert Panel undertakes reviews and considers emerging evidence to assess whether medicines remain eligible for inclusion on the program as well as the ongoing suitability of testing requirements. In addition to formal reviews, the Expert Panel also considers emerging evidence that may alter a medicine's eligibility for the program. This may include, for example, new evidence about condition prevalence which may mean that a condition no longer meets the program's definition of 'ultra-rare'; the Expert Panel considered such evidence for Fabry disease at its June 2022 meeting and determined that Fabry disease no longer met this criterion. Further consideration may be required as to how these additional functions would be managed if recommendations on LSDP listings become the sole responsibility of the PBAC, if the program was retained outside the current PBS framework.

For example, the Expert Panel currently reviews all LSDP medicines 24 months after listing in order to better understand the real-world use of a medicine by comparing the actual performance and use of the medicine to the recommendations and expectations at the time of listing. Consideration will be required as to whether these reviews should be included in existing PBS review arrangements, or whether reviews of LSDP medicines should remain distinct from PBS processes (and if so, which committee would have responsibility).

Conclusion

The Expert Panel welcomes the opportunity to provide input to the important work of the Reference Committee through the HTA Review. The Expert Panel emphasises the importance of the LSDP in providing more equitable access to those Australians with serious ultra-rare conditions causing very high lifelong health and cost burdens, by providing a mechanism to supply very high cost and clinically effective medicines in Australia. The Expert Panel would support changes to current assessment processes that would reduce duplication in assessment, and supports the intent of the proposals. However, the Expert Panel would welcome further detail on implementation considerations for these proposals in the HTA Review's final report, noting that the details of implementation of any agreed recommendations will be a matter for Government.