

Ensuring the future sustainability of the Life Saving Drugs Program (LSDP)

Agreement between the Government and Medicines Australia (MA)

Purpose:

The LSDP delivers access to high-cost lifesaving medicines for eligible patients with chronic progressive rare diseases which have not been recommended to be listed on the PBS on the basis that the medicines are not cost-effective. The parties acknowledge that the purpose of this Agreement is to facilitate and promote cooperation between the parties in respect to ensuring the future sustainability of the LSDP.

This Agreement is a commitment by the Government and MA to:

- Ensure the future sustainability of the LSDP
- Deliver more certainty for the medicines industry through a transparent and rigorous process for medicine listing on the LSDP
- Encourage companies to continue to bring innovative, life-saving medicines to Australia at a cost-effective price

The agreement is underpinned by the shared principles of:

- **Stewardship** of the Australian health system and a responsibility for its ongoing sustainability
- **Patient access** to clinically effective medicines for chronic progressive rare diseases
- **Improved value** of medicines available on the LSDP that enable ongoing sustainability of the program
- **Stability and certainty** for the investment in medicines for rare diseases, including recognition of the role that transparent and streamlined processes play in encouraging investment
- **Transparency and efficiency** of processes for listing medicines on the LSDP and for subsequent reviews of medicines

Statement of Intent for the Government

The Government recognises the challenges inherent in reimbursing medicines for rare diseases, including the high costs of medicine development, small patient populations, sparse efficacy data, and growing demand for ongoing access to high-cost life-extending treatments. It also recognises the pivotal role of the sector in delivering these breakthrough therapies that represent important advances in medicine and improve the lives of Australians.

Certainty and transparency

The Government commits to working with MA to improve the certainty and transparency of listing medicines on the LSDP. This work may include fair and transparent cost recovery arrangements that support improvements to the evaluation and administration of LSDP medicines.

Establishment of Expert Panel

An Expert Panel will be established that will provide assistance and advice to the Commonwealth Chief Medical Officer (CMO) in assessing rare disease medicines seeking listing on the LSDP. The six member expert panel will include suitably qualified experts including a consumer representative and an industry nominee and will meet up to three times per year.

Sponsors will continue to have the opportunity to nominate a clinical expert to attend meetings supporting the PBAC decision making process (e.g. stakeholder meetings), as per current PBAC arrangements. A member of the Expert Panel may also be nominated to attend these meetings.

Consideration of medicines by the Expert Panel will be sufficiently flexible to consider the needs of, and benefits to, patients living with rare diseases and their carers. The Panel may provide advice to the CMO on a range of matters including clinical need, clinical effectiveness, managed access arrangements, further evidence collection, patient caps, or risk sharing arrangements.

Explanatory materials and guidance

The Government commits to developing explanatory materials to support the existing LSDP criteria to include a rare diseases definition, and specify that listed medicines must extend lifespan, including through substantial reduction of the level and duration of disability that would otherwise lead to a significant reduction in life expectancy.

To support the Expert Panel advice and CMO decision, guidance will be developed to assist sponsors in preparing an application to list a rare disease medicine on the LSDP. The LSDP program guidance will be developed in line with the 29 January 2018 Government Response to the Review of the LSDP and will also consider the needs of the Expert Panel in its advisory role to the CMO.

Timelines

The Government commits to working with MA to develop guidance, in line with the Government response to the LSDP review, for a clearly defined and transparent process and associated timelines for consideration of listing on the LSDP by the Expert Panel and the CMO. This will provide certainty to sponsors, as well as ensuring access to treatment for people with rare diseases is not unnecessarily delayed.

All new medicines for rare diseases will need to be considered first by the PBAC for potential listing on the PBS. If the PBAC considers the new medicine clinically effective but not cost effective, the sponsor will then be able to apply for inclusion on the LSDP.

Statement of Intent for Medicines Australia (MA)

Medicines Australia recognises that stewardship is important to ensure the long term sustainability of the LSDP, to ensure costs paid are reasonable and that the Government only pays for medicines when clinical performance expectations are met.

Medicine reviews

MA will support reviews of existing medicines on the LSDP, as well as reviews of new medicines 24 months after initial listing on the LSDP to assess usage, financial costs and any other information requested for collection at the time of listing. This will ensure that use and performance of the medicine is in line with the expectations at the time of listing.

Data collection

Where the Expert Panel advises the CMO that it would be practical to confirm the benefits of treatment in terms of extending lifespan or reducing the level and duration of disability that leads to reduction in life expectancy, de-identified program data will be provided to support the Expert Panel's consideration. Sponsors will also provide additional information to support the Expert Panel's considerations.

Price reductions

In recognition that the reforms to the existing LSDP will improve patient access to medicines, Medicines Australia supports in-principle the application of pricing policies that mirror existing PBS pricing policies (including ministerial discretion). The parties recognise and expressly agree that prices of LSDP products are a matter solely between the Government and each sponsor under bilateral arrangements between them.

The application of PBS-like pricing policies will commence from 1 April 2019 and will be negotiated with individual sponsor companies with medicines on the LSDP. PBS-like pricing policies will cease after the final reduction anniversary in 2022.

Pricing policies will be applied as per, and in line with, PBS pricing policies on an administrative basis.

Deeds of Agreement

Deeds reflect individual company legal agreements with the Commonwealth. There may be revised pricing arrangements in current and future deeds, which must be

negotiated solely between the Commonwealth and each individual sponsor company with a medicine on the LSDP under bilateral agreements between them.

Existing deeds may be revised through this bilateral negotiation process to incorporate pricing recommendations made to the CMO by the Expert Panel following medicine reviews, however that will be a matter for agreement between the Commonwealth and the sponsor.

Duration of Agreement:

The commitments outlined in this Agreement between the Government and MA are valid for the duration of the Agreement. This Agreement will operate from 1 July 2018 to 30 June 2022.

Any changes to the terms of this Agreement need to be agreed by both parties subject to consultation. This document does not create legal obligations on either party.