



Australian Government

Australian Government response to the

**Post-market Review of the Life Saving Drugs
Program**

Introduction

The Australian Government's Life Saving Drugs Program (LSDP) provides subsidised access for eligible patients with rare and life-threatening diseases to essential and very expensive medicines. The program initially operated as Act of Grace payments in 1995 and later through the establishment of a more formal LSDP. Persons with these rare diseases often require medicines that have a very high cost per patient. These medicines often fail to meet the comparative cost effectiveness criteria required for Pharmaceutical Benefits Scheme (PBS) funding. The LSDP provides eligible patients with access to these life-saving medicines at no expense to the patients or their families.

In 2016-17, the LSDP assisted 393 patients at a cost of \$116 million. In the past six years (2011-12 to 2016-17) the number of patients assisted by the program has grown over 65 per cent.

The LSDP currently funds medicines to treat nine serious rare and life threatening medical conditions. These are Fabry disease, Gaucher disease Type 1, Mucopolysaccharidosis Types I, II, IVA and VI, Pompe disease (Infantile-onset, Juvenile Lateonset or Adult Late-onset), Paroxysmal Nocturnal Haemoglobinuria, and Hereditary Tyrosinaemia Type I.

The 13 currently funded medicines are:

- Replagal[®] (agalsidase alfa)
- Fabrazyme[®] (agalsidase beta)
- Cerezyme[®] (imiglucerase)
- VPRIV[®] (velaglucerase)
- Eleyso[®] (taliglucerase)
- Zavesca[®] (miglustat)
- Aldurazyme[®] (laronidase)
- Elaprase[®] (idursulfase)
- Naglazyme[®] (galsulfase)
- Myozyme[®] (alglucosidase alfa)
- Orfadin[®] (nitisinone)
- Soliris[®] (eculizumab)
- Vimizim[®] (elosulfase alfa)

Australia, like other developed countries, faces growing pressure to fund increasing numbers of expensive specialised medicines for rare diseases, often through fast tracked and rapid assessment processes and listing decisions. This can give rise to pressure to approve and reimburse/fund the medicine, without a full understanding of the medicine's effectiveness and whether the benefit to the patient is commensurate with the price paid for the medicine (cost effectiveness).

The Government commissioned an independent expert review to explore a range of matters associated with these rare (and also referenced as ultra-rare) diseases including questions of access and equity, value for money and the future administration of the program.

Background to the Post Market Review

On 9 April 2014, the then Minister for Health, the Hon Peter Dutton MP, announced the Government would review the Life Saving Drugs Program (LSDP). The Review would be undertaken by an independent expert panel and report to the Australian Government.

Following public consultation in late 2014, the terms of reference for the Review were established. These were to:

1. Review the clinical effectiveness and safety of medicines currently subsidised through the LSDP.
2. Review the emerging clinical treatments and diseases, including those that identify sub-groups by molecular target, which could potentially seek subsidisation through the LSDP in the future.
3. Conduct an international comparison of subsidisation of drugs for rare diseases and the definitions for a rare/ultra-rare disease.
4. Compare the subsidisation and equity principles of the Pharmaceutical Benefits Scheme (PBS) and the LSDP.
5. Assess the value for money of the medicines subsidised on the LSDP by evaluating the benefit of each drug's treatment outcomes, including in terms of quality of life achieved through the programme and their cost.
6. Review the administration of the LSDP including the Guidelines with which the programme is administered for each condition and assess alternative administrative systems.
7. Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally.

The Review was undertaken within the broad principles of the PBS Post Market Review framework.

The independent Expert Reference Group (ERG) was chaired by Professor Andrew Wilson (Director of the Menzies Centre at the University of Sydney, and Chair of the PBAC since July 2015), and included expert advice including in the areas of specialist clinicians, medical ethics, health economics, and consumer input.

Membership of the Life Saving Drugs Program Reference Group

- Professor Andrew Wilson – Chair
- Professor David Sillence – Clinical Expert
- Professor David Isaacs – Clinical Expert
- Professor Anne Tonkin – Clinical Expert
- Professor Jane Hall – Health Economist
- Professor Paul Komesaroff – Medical Ethicist
- Mrs Lesley Murphy – Consumer Representative, Rare Voices Australia
- Ms Ainslie Cahill – Consumer Representative, Consumers Health Forum; CEO, Arthritis Australia

The ERG was assisted in its deliberations through the provision of additional technical support:

- A health technology assessment report by the University of Adelaide Health Technology Assessment group (terms of reference 1-3, 5 and 7), (the Evaluator's report);

- A research report into consumer perspectives undertaken by the Consumers' Health Forum of Australia (terms of reference 4 and 7); and
- A report on the current administration of the Program provided by the Department of Health.

As a result of extensive stakeholder engagement, interested parties made 90 submissions to an Issues Paper that was issued in April 2015. The Consumers' Health Forum also convened two consumer workshops in Sydney and Melbourne to discuss consumer perspectives on the program.

The Review considered this information and the submissions from the public and made a series of comments and recommendations to Government.

The Government's Plan for reform

The Government will continue to support access to high cost life savings medicines through the Life Savings Drugs Program (LSDP).

The Government welcomes the findings of the Review and acknowledges the importance and need to continue a program that enables access for Australians with rare life threatening diseases to funding for lifesaving medicines that are safe and clinically effective.

Targeted consultations will occur following the release of the report as part of implementing the new arrangements for the LSDP.

LSDP Review Recommendation 1

The Commonwealth Government should continue to enable access to and provide funding for medicines to treat Australians with rare diseases, where those medicines have been evaluated for safety, efficacy and clinical effectiveness.

LSDP Review Recommendation 2

Medicines currently included on the Life Saving Drugs Programme (LSDP) should be grandfathered to a new Medicines for Rare Diseases Programme (MRDP) to ensure existing and new patients who meet eligibility criteria and who continue to benefit from receiving treatment for diseases currently funded under the LSDP will continue to be supported.

LSDP Review Recommendation 3

The LSDP should be transitioned from a standalone programme and be formally established as a special programme under section 100 of the National Health Act 1953, mirroring other section 100 programmes such as the Highly Specialised Drugs Programme, to benefit from existing structures, processes and systems currently within the Pharmaceutical Benefits Scheme.

LSDP Review Recommendation 4

The new programme should be known as the Medicines for Rare Diseases Programme. Eligibility criteria for consideration of listing under the new programme are proposed based on the current LSDP criteria. These new criteria should be reviewed in two years or after the first four submissions have been assessed using the new criteria (whichever comes first).

LSDP Review Recommendation 5

There is a need when considering the value of medicines for rare diseases to consider matters beyond cost-effectiveness. These principles are already embedded in the approach used by the PBAC in its decision making but this would benefit from being more transparent.

LSDP Review Recommendation 6

Consideration should be given to enhancing the medicines submission process for rare disease therapies by adopting a collaborative multi-stakeholder approach early in the assessment cycle, before the medicine submission is formally submitted for consideration by the PBAC.

LSDP Review Recommendation 7

Rare disease' should be defined for the purpose of the Medicines for Rare Diseases Programme.

Government Response

The Australian Government will retain and improve the LSDP, drawing upon recommendations 1 through 7 of the Review. This will ensure: eligible patients retain ongoing access to medicines currently available through the LSDP; a pathway to consider new medicines which includes fit-for-purpose clinical effectiveness and cost effectiveness assessment; and the future integrity and sustainability of the program.

The changes will include:

- the adoption of a rare diseases definition, that being a disease prevalence of 1:50,000 people or less in the Australian population (around 500 people). This is in line with the current LSDP prevalence rates.
- developing explanatory materials to support the criteria to specify that lifesaving medicines are those that extend lifespan, including through the measurement of substantial reduction to the level and duration of disability, which will lead to a significant increase in life extension.
- implementation of more transparent and rigorous clinical efficacy and cost effectiveness assessment of medicines, delivered through the establishment of an expert panel which will provide advice and assistance to the Commonwealth's Chief Medical Officer (CMO).
- introduction of a mechanism where medicines listed on the LSDP will be subject to a review of usage and financial costs after 24 months, ensuring use and performance of the medicine is in line with the recommendations and expectations at listing. Similar reviews will be undertaken on all existing LSDP medicines over the first two years from the commencement of the new program.
- the negotiated application of pricing policies to new and existing medicines on the LSDP, as per those applying to Pharmaceutical Benefits Scheme (PBS) listed medicines.
- streamlining administration of the LSDP, and implementing cost recovery arrangements from sponsors for listing considerations and management of their agreements.

These improvements to the LSDP deliver certainty to patients and stakeholders.

LSDP Review Recommendation 8

A small number of centres of clinical expertise in rare diseases should be established. These should incorporate state-based clinical advisory committees, with the larger states networking with smaller states or territories.

The Australian Government supports the intent of this recommendation. The COAG Health Council (CHC) and its advisory body, the Australian Health Ministers' Advisory Council (AHMAC), provide a mechanism for the Australian Government, the New Zealand Government and state and territory governments to discuss matters of mutual interest concerning health policy, services and programs. The Australian Government will look for opportunities to discuss this recommendation in these fora.

LSDP Review Recommendation 9

The Department of Health should support the development of a fit-for-purpose data collection framework and require sponsors of medicines for rare diseases to collect the data necessary to support initial and ongoing evaluation of medicines funded under the proposed Medicines for Rare Diseases Programme.

This recommendation is accepted in-principle.

Medicine sponsors will be required to support the initial listing decision and its ongoing inclusion in the LSDP. Where it is considered practical, the latter may include a sponsoring company undertaking an ongoing program to confirm or clarify the benefits of its medicine within a reasonable period of time, through a 'managed access' arrangement.

Medicines listed on the LSDP will be subject to a review of usage and financial costs after 24 months, ensuring use and performance of the medicine is in line with the expectations and recommendations applied at listing. Similar reviews will be undertaken on all existing LSDP medicines over the first two years from the commencement of the new program.

LSDP Review Recommendation 10

The reference group considers some matters out of scope for the LSDP Review but recommends that further consideration be given to these matters raised by stakeholders.

The Australian Government notes that a range of issues were identified which fell outside the scope of the Review.

These issues may be considered during and after the establishment of the new Program.