Information for patients relating to the Life Saving Drugs Program (LSDP)

1. What is the LSDP?

The Australian Government provides very expensive and life-saving medicines for rare and life-threatening medical conditions through a program called the Life Saving Drugs Program (LSDP).

The day to day administration of the LSDP is managed by the Commonwealth Department of Health. A patient's treating physician applies to the Department to have their patient accepted onto the program. The Department liaises directly with the supplier of the medicine to have the medicine shipped to an agreed location (usually a hospital pharmacy) to be dispensed to the patient.

2. Is this program part of the Pharmaceutical Benefits Scheme (PBS)?

The LSDP is separate to the PBS. The medicines within the LSDP do not fit the PBS due in part to the high cost of the medicine. The Australian Government considers these medicines support Australians who need them, and therefore supply the medicines through the LSDP without the patient incurring a co-payment.

Please visit the PBS webpage for more information on the PBS.

3. How do I access a medicine on the LSDP?

The LSDP funds 14 medicines for 9 rare diseases. To be eligible to access one of these medicines, you must first be diagnosed with one of the specific conditions by a rare disease specialist, who can then apply to the LSDP on your behalf by completing an initial application form, with supporting test results that confirm the diagnosis. Each condition has its own guidelines and application forms. These are available on the LSDP website.

A patient must meet the following to receive an LSDP medicine:

1. Satisfy the relevant criteria for treatment with the medicine, as detailed in the relevant LSDP Guidelines.

2. Have certain clinical tests performed by your physician at regular intervals so that the medicine can be assessed for effectiveness. Alternatively, the patient must have an acceptable reason not to participate in these tests.

3. Not be suffering from any other medical condition, including complications of the primary condition that might compromise the effectiveness of the medicine.

4. Be a permanent Australian resident who qualifies for Medicare.

Once a year, each patient on the program is assessed to confirm they are still eligible. The physician must submit a reapplication for each patient by 1 May each year to ensure that their patient remains eligible to receive subsidised treatment. Each condition has specific evidence that needs to be provided by the physician and must show that the patient is either clinically improving; or that their condition is stable.

A patient must also adhere to their treatment regime to ensure they continue to be eligible for ongoing treatment.

An assessment of eligibility is made considering the advice of the physician and the natural course and stage of the disease that the patient suffers from. In some cases, exceptional circumstances will also be taken into consideration.

4. Why has my physician not heard back regarding my application?

Once the Department has received all relevant documentation for an application to access LSDP medicines, the physician will be notified as soon as possible whether their patient has been approved.

The approval process can take several days. A number of eligibility checks must be undertaken, for example:

- the form has to be checked to see if it is completed correctly and all the required information submitted. The Department cannot process an incomplete application;
- the individual test results need to be compared to the specifications in the disease guidelines;
- confirmation of Medicare eligibility must be sought from the Department of Human Services;
- the documentation must be assessed to determine that all necessary eligibility criteria have been met;
- the dose prescribed by the physician needs to be confirmed for appropriateness based on the patient characteristics (such as age or weight);
- the number of vials/tablets required for each dose needs to be calculated to determine the annual cost of treating the patient; and
- the information is collated and approval must be sought from the Department approval delegate.

New patient applications are prioritised and are actioned as soon as possible. Thirty days is the maximum amount of time that it could take for the physician to be notified of the outcome of their correctly completed application.

5. Can I still travel while on the program?

Yes.

In accordance with the LSDP guidelines, physicians are required to authorise any travel their patients wish to undertake that would affect their access to their prescribed treatment.

If you wish to travel while on the program, you should discuss with your treating physician how to manage your treatment over the travel period. You should be aware that out-of-pocket expenses may be incurred when travelling overseas.

After a treatment regime is agreed, it is the treating physician's responsibility to:

a) arrange for a treating physician and dispensing pharmacy at the new location including an overseas location; and
b) provide the LSDP with the relevant travel details (ie. location, travel period, treating physician and pharmacy, any other relevant details).

6. How does a medicine get funded on the LSDP?

Before a medicine can be used in Australia, it must first be approved by the **Therapeutic Goods Administration (TGA)**. The TGA is part of the Australian Government Department of Health, and is responsible for regulating all therapeutic goods including prescription medicines, vaccines, sunscreens, vitamins and minerals, medical devices, blood and blood products.

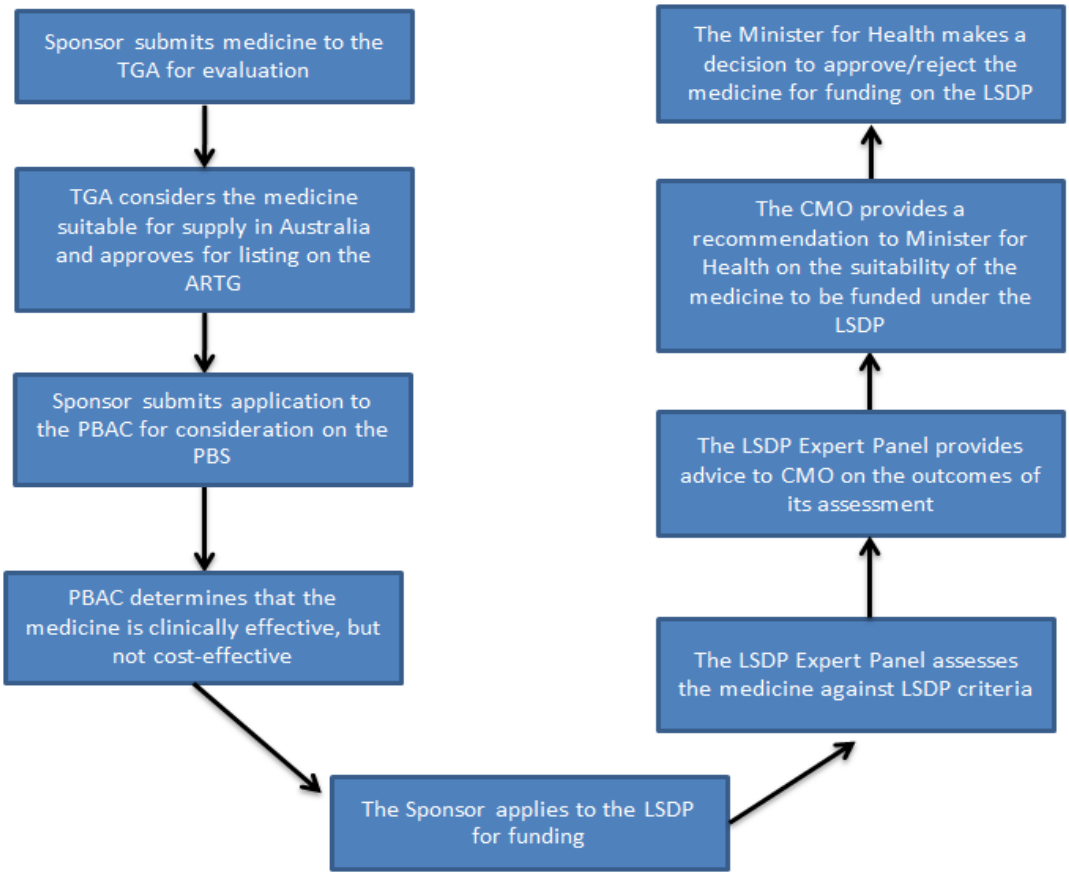
The TGA makes sure Australians have access to therapeutic goods that are effective, safe to use and of good quality - meaning that they have been properly tested, formulated and manufactured to certain standards.

Before being considered for the LSDP, medicines must first be considered by the **Pharmaceutical Benefits Advisory Committee (PBAC)** for funding under the Pharmaceutical Benefits Schedule. This is the Government's preferred mechanism for funding medicines, and a number of medicines for rare diseases are funded on the PBS. Patients are encouraged to provide online comments to the PBAC as part of the process.

PBAC will provide a recommendation to the Commonwealth Minister for Health on the medicine's suitability for the PBS. If the committee finds a medicine to be clinically effective but not cost-effective (i.e. the medicine works but is too expensive for the PBS), the sponsor can apply to the **LSDP** for funding.

An **Expert Panel** for the LSDP has been established to assess whether the medicines seeking funding on the LSDP meet certain criteria. This Panel provides assistance and advice to the Commonwealth Chief Medical Officer (CMO) in his development of a recommendation to the Commonwealth Minister for Health. The Expert Panel includes qualified experts in health technology assessment, including a consumer representative, an industry nominee, a health economist and clinicians. The Panel meets three times per year, usually February, June and October, after the PBAC meets.

The flowchart below provides an overview of the funding process.



7. Can patients/carers provide input to the LSDP Expert Panel when they consider whether a new medicine should be funded on the program?

Yes.

Patients, their carers, and their treating physicians are central to the assessment of new medicines, particularly when considering medicines for rare diseases. Understanding patients' perspectives is essential to the Panel's consideration. All consumer comments provided to the PBAC during their consideration of a new medicine for the PBS will also be available to the LSDP Expert Panel if the sponsor of the medicine applies to the LSDP. Once the agenda for the upcoming Expert Panel meeting is published on the LSDP webpage, any interested parties are welcome to provide their input directly to the LSDP Expert Panel Secretariat via email.

Further information on the process of new applications can be found in the LSDP Guidance document on the LSDP webpage.

8. Why are there reviews of LSDP medicines?

Reviews are necessary because when a medicine is first funded there is still uncertainty about the evidence for the real world effectiveness of the medicine or its success in extending life. The reviews also help to ensure the sustainability of the program.

The reviews aim to:

- develop a better understanding of the real-world use of a medicine;
- review and confirm the clinical benefits achieved through medicine use;
- ensure the ongoing viability of the program;
- ensure testing and access requirements and the price paid for the medicine remain appropriate and;
- improve our understanding of whether or not the medicine significantly extends life.

9. *How will the review of new medicines after two years of funding affect my access to a medicine?*

When a medicine is first funded on the LSDP, the sponsor company and the Department agree on the patient data that will be collected to inform the review that will occur after two years of funding. The primary aim of this review is to analyses whether the actual outcomes are the same as the predicted outcomes at the time of funding. The review evaluates the data collected annually from LSDP patients as well as any additional data provided by the sponsor company or that has been published internationally. Consumers and clinicians will have the opportunity to provide input to the review. A review report is completed by the Department for consideration by the Expert Panel, along with any responses made by the medicine's sponsor, clinicians and stakeholders. The review is expected to take place over four to eight months.

Following the outcome of the review, the Department and the sponsor may renegotiate who should access the medicine to ensure that subsidisation continues at prices that reflect the effectiveness of the medicine.

All patients will continue to access their medicine on the program during the course of the review, and any outcomes from the review will be published on the LSDP website.

Further information on the review process can be found in the LSDP Guidance document on the LSDP webpage.

10. *How is the review of existing LSDP medicines different to the review of new medicines after the first two years of funding?*

When the new arrangements for the LSDP took effect on 1 July 2018, they included conducting reviews on all the then current medicines funded on the LSDP. Some medicines have been listed on the LSDP since its inception and these reviews aim to ensure use and performance of the medicine is in line with the recommendations and expectations at the time of funding, as well as understand any changes in best clinical practice that may have occurred.

Patients/carers will have multiple opportunities to provide input to the Expert Panel for the reviews of existing LSDP medicines. Written input is collected from the time the Panel considers what the main issues are that need to be assessed until the draft report is assessed by the Panel. As the reviews for each disease group are staggered over the course of two years, input is collected at different times. The timelines and deadlines are announced on the LSDP webpage and input can be provided to the LSDPEP@health.gov.au inbox.