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. All LSDP medicines have been considered but not recommended for the PBS due in part to the high cost of the medicine. The Australian Government considers these medicines benefit Australians who need them, and therefore supply the medicines through the LSDP without the patient incurring a co-payment.

Please visit the [PBS webpage](https://www.pbs.gov.au) for more information on the PBS.

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

The LSDP currently funds 16 medicines for 10 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 eligibility guidelines and application forms. These are available on the [LSDP website](https://www.health.gov.au/).

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. Undergo 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 experienced by the patient. In some cases, exceptional circumstances will also be taken into consideration.

4. Will I pay for the supply of my medicine?

The Department expects all patients receiving medicines under the LSDP to be categorised as public patients. Public hospitals are jointly funded by the Commonwealth Government and State/Territory Governments under the National Health Reform Agreement (NHRA) and the National Health Funding Pool. Importantly, all services in public hospital to eligible patients are to be provided free of charge as per Clauses 4 and 5 of the NHRA. Hospitals (whether public owned or private providing public services) are also not to charge for any services provided to public patients. The Government’s commitment to these clauses was reaffirmed in the latest addendum to the NHRA.

For patient who collect their LSDP medicine directly from a pharmacy for use outside of the hospital, the pharmacy may provide you with an esky. The pharmacy may choose to charge patients for the provision of this cooling equipment.

5. 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 Services Australia;
• 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.

6. **Why do I have to have clinical tests done each year?**

As part of the annual reapplication process, each treating physician must submit certain test results for their patients. This allows the LSDP to confirm that patients are satisfying the relevant criteria for treatment with the medicine, as set out in the relevant LSDP Guidelines for each medicine. Each disease has different tests that should be undertaken each year. The collection of this data is agreed to when a patient signs the consent form as part of the annual reapplication process.

If you have concerns about why certain tests are needed or how often, please discuss this with your treating physician. If your physician has any questions or concerns, they can direct them to the LSDP team via email or phone.

7. **Can I still travel while on the program?**

Yes.

If a patient wishes to travel while on the program, they must notify their treating physician of the dates of travel.

It is then the treating physician responsibility to:

a) Authorise travel by contacting the LSDP;
b) Arrange for a treating physician at the new location including an overseas location; and
c) Ensure the patient is aware that out-of-pocket costs may be incurred.

8. **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.
9. 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.

10. How long will it take for a new medicine to be added to the LSDP?

When a potential new medicine is being considered for listing on the LSDP, it is important that the public are kept updated on the medicine’s progress through the process. It is important for the public to understand the stages of the LSDP process.
From the flow diagram above, the medicine must first be considered by the PBAC and found to be clinically effective but fail to meet the cost effectiveness criteria. The sponsor can then apply for subsidisation through the LSDP.

The PBAC outcome for each medicine application is released on the PBAC Outcomes webpage approximately one month after each PBAC meeting. This will outline if the medicine has been recommended for the PBS or not. The agenda for each Expert Panel meeting is uploaded on the LSDP webpage one month before the Expert Panel meeting date. The public can see which new medicines will be considered by the Expert Panel for funding on the LSDP. On the LSDP New Medicine Application webpage, there is a flow chart outlining the process and timings for a new medicine.

The CMO will consider advice from the LSDP Expert Panel on any new medicine for the LSDP, and provide a recommendation on the suitability of a medicine for funding under the LSDP. The CMO requires two to six weeks to provide a recommendation to the Minister for Health. If the Minister agrees with a positive recommendation, pricing negotiations begin with the sponsor. During such time, no information regarding the medicine can be released to the public due to commercial-in-confidence and budget-in-confidence caveats. The time it takes for negotiations to conclude varies for each medicine.

Following Government agreement to list a new medicine on the LSDP, all necessary information and paperwork for doctors will be added to the LSDP Information for patients, prescribers and pharmacists webpage. A public announcement is also usually made.

11. 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.
12. 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.

13. 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.