Australian Government
Department of Health and Aged Care

Life Saving Drugs Program (LSDP) Review of Medicines for Hereditary Tyrosinaemia (HT1) and Expert Panel recommendations

Consumer Summary

Purpose of the LSDP Review

The LSDP provides funding for medicines to treat people living with rare and life-threatening diseases. Within the LSDP a rare disease is defined as one which occurs in 1 per 50,000 people or fewer.

The LSDP Expert Panel (the Panel) has recently conducted a review of medicines the LSDP funds to understand how people now use these medicines compared to when they were first put on the program. It also looked at how the medicine benefits patients and whether the right tests are used.

Potential changes that the Government can make to the funding criteria are also considered.

The reviews had seven Terms of Reference (ToRs). These ToRs cover the following issues:

1. **Disease Prevalence:** How many people have the disease in Australia (called disease prevalence) and can it still be categorised as a rare disease under the LSDP?
2. **Treatment Guidelines:** How is the disease being treated currently compared to the LSDP treatment guidelines for access to the medicine, such as eligibility for treatment and testing requirements?
3. **Medicine Effectiveness and Safety:** For a medicine to be suitable for the LSDP, it must help people to live longer. Does the medicine enable this, and how does this compare with other medicines, based on reports provided on people receiving LSDP-funded medicine and international reports?
4. **Treatment Outcomes:** What do people receiving LSDP-funded medicine consider its most important benefit to their lives and how does this compare with the patient-reported information currently required to be collected for the LSDP?
5. **Value for Money:** Does the benefit of treatment (e.g. years of life gained) reflect the estimate of this at the time the medicine was first approved for LSDP funding?
6. **Drug Usage:** Is there any wastage in the way the drug is stored, dosed and dispensed and could this be improved?
7. **Future Treatments:** Are there new medicines being developed and/or soon to become available which may broaden treatment choices?

The Panel advised the Chief Medical Officer (CMO) of the results of the review for each medicine. The CMO then recommended certain actions to the Minister for Health and Aged Care.

The LSDP HT1 Review

At the time of the review, nitisinone (brands Orfadin® and Nityr®) was the only medicine for HT1 listed on the LSDP. Orfadin® was listed on 1 June 2016. The generic brand, Nityr®, was not included in this review because it was put on the LSDP later (1 May 2019).

The following summarises the advice of the Panel following the HT1 Review for each of the seven considerations listed above.

1. Disease prevalence

The prevalence of HT1 in Australia was fewer than 1 in 50,000 people and it is therefore suitable for the LSDP. The Panel noted that if all newborn babies in Australia were tested for HT1, as is done in some overseas countries, more HT1 patients may be found. Those patients might then be able to start treatment sooner.

2. Treatment guidelines

Criteria used by the LSDP to decide if someone is eligible for treatment match those used overseas. The Panel noted that if a patient has had a liver transplant then they probably should not take nitisinone. This is because it is not known if nitisinone would work or be safe to use after a liver transplant. The Panel recommended this change should be made to the LSDP HT1 guidelines.

3. Medicine effectiveness and safety

People appear to live longer when receiving nitisinone for HT1, compared to people not receiving treatment. This medicine therefore remains suitable for the LSDP.

The Panel noted that some patients with HT1 have neurocognitive impairment (problems with memory, language, thinking or judgement). It is not known if this is caused by nitisinone because there is not yet enough information available.

4. Treatment outcomes

The LSDP has not been receiving all relevant test results for new and ongoing applications from treating physicians. To better understand the benefit of treatment, the LSDP should review:

- how to collect patient outcomes information and what is collected; and
- the way this information is analysed.

The LSDP should also make it easier for treating physicians to provide the patient data required for applications. This will improve the everyday work of the program and future medicines reviews.

The Panel also wanted treating physicians to know that they can contact the LSDP at any time to change a patient's nitisinone dose.

5. Value for money

People treated with nitisinone appear to live longer and have an improved quality of life. However, the cost of the medicine remains high. It was recommended that the price that the Government pays for nitisinone be reassessed and discussed with the drug company. The goal is to improve the value for money so the LSDP can provide high value medicines now and in the future.

6. Drug usage

The Panel noted that there were fewer people taking nitisinone than was expected when it first went on the LSDP. This has meant the Government has spent less on nitisinone than expected.

7. Future treatments

The Panel noted that drug companies are developing new treatments for HT1. However, these are not expected to be ready for patients in the near future. The Panel noted that if all newborn babies in Australia were tested for HT1 then more people with the disease may be found. Those patients may be able to start treatment with nitisinone sooner. The Panel also noted that the LSDP was not responsible for newborn testing.

Next Steps

The Minister for Health and Aged Care has agreed to these recommendations. The implementation of these recommendation is currently being considered and progressed by the Department in consultation with sponsors, treating doctors and patient advocacy groups.