Life Saving Drugs Program (LSDP) Review of Medicines for Fabry disease 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 medicines benefit 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 Fabry Disease Review

There are three medicines for Fabry disease listed on the LSDP:

- agalsidase alfa (Replagal®)
- agalsidase beta (Fabrazyme®), and
- migalastat (Galafold®).

The LSDP Fabry Disease Review considered the two enzyme replacement therapies (ERTs), agalsidase alfa (Replagal®) and agalsidase beta (Fabrazyme®). Both received LSDP funding for Fabry disease on 1 July 2004. Migalastat was not included in this review because it was added to the LSDP later (2018).

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

1. Disease prevalence

The prevalence of Fabry disease in Australia was fewer than 1 in 50,000 people and it is therefore suitable for the LSDP. An increasing number of people are getting tested (such as family members of people who have the disease) and therefore, more people are likely to receive a diagnosis of Fabry disease and may need an LSDP medicine. The Panel recommended that the Government should review the prevalence of Fabry disease in the next five years.

2. Treatment guidelines

Criteria used by the LSDP to decide if someone is eligible for treatment do not match those used overseas. The Panel recommended that the drug companies that provide LSDP medicines should apply to change the eligibility criteria. The Panel also recommended that the LSDP review the medical tests required for adult and paediatric patients to be eligible for the program. This review needs to have input from treating physicians managing Fabry disease patients.

3. Medicine effectiveness and safety

People appear to live longer when receiving either agalsidase alfa (Replagal®) or agalsidase beta (Fabrazyme®) for Fabry disease, compared to people not receiving treatment. These medicines therefore remain suitable for the LSDP. The Panel also found that there was nothing to suggest that one medicine was better than the other.

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:

1. how to measure pain and quality of life
2. how to collect patient outcomes information and what is collected; and
3. 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.

5. Value for money

People treated by these medicines appear to live longer and have an improved quality of life. However, the cost of these medicines remains high. It was recommended that the price that the Government pays for these medicines be reassessed and discussed with the drug companies. 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 dose of Fabry medicines is based on the person's weight. Sometimes, more than the prescribed dose is given due to the size of the medicine vial. For example, if the dose equates to five and a half vials, six vials would be given. The Panel did not disagree with whole vials being given to patients but recommended that the Government should look at what it pays for this and discuss it with the drug companies. Other patients cannot use an open vial and it cannot be saved.

7. Future treatments

The Panel noted that drug companies are developing new medicines for Fabry disease. However, these new treatments are not expected to be ready for patients in the near future.

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.