



# Life Saving Drugs Program (LSDP) Review of Medicines for Mucopolysaccharidosis Type I (MPS I) and Expert panel recommendations

## Consumer summary

### Purpose of the LSDP Review of its funded medicines

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 which it 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 review of each of the LSDP-funded medicines has seven Terms of Reference (ToR). These ToRs cover the following issues:

1. **Disease Prevalence:** How many people have the disease in Australia (called disease prevalence) and does this enable it to 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 current medicine enable this, and how does this compare between medicines, 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 there be improvements in this?
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 MPS I Review

At the time of the review, laronidase (Aldurazyme®) was the only medicine for MPS I listed on the LSDP. It was listed on 1 August 2007.

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

### 1. Disease prevalence

The prevalence of MPS I in Australia was fewer than 1 in 50,000 people and is therefore suitable for the LSDP. The Panel recommended that the Government should review the prevalence of MPS I in the next five years.

### 2. Treatment guidelines

Criteria used by the LSDP to decide if someone is eligible for treatment does not match those recommended by MPS I treating physicians. The Panel recommended that the drug company that provides laronidase should apply to the Panel to change the eligibility criteria. The Panel also recommended that the LSDP review the medical tests required for patients to be eligible for the program. This review needs to have input from treating physicians managing MPS I patients.

### 3. Medicine effectiveness and safety

The Panel noted that there is not enough information available to confirm if laronidase improves survival in MPS I patients. For most MPS I patients, laronidase appears to either slow the effects of the disease or keep it stable. The Panel noted that this was an important result and that laronidase therefore remains suitable for the LSDP.

### 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 collect patient outcomes information and what is collected; and
2. 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 with laronidase 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 laronidase 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 recommended that the drug company for laronidase (Aldurazyme®) make home infusions available for patients to enable the LSDP to allow the medicine to be given at a patient's home.

## 7. Future treatments

The LSDP does not allow patients to receive an LSDP medicine if they are participating in a clinical trial (and receiving other medicines or treatments). As part of their recommendations, the Panel advised that patients who are in a clinical trial can remain on the LSDP.

### 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.