



# Life Saving Drugs Program (LSDP) Review of Medicines for Pompe 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 Pompe Disease Review

At the time of the review, alglucosidase alfa (Myozyme®) was the only medicine for Pompe disease listed on the LSDP. It was listed for infantile onset Pompe disease (IOPD) on 1 February

2010; for juvenile onset Pompe disease (JOPD) on 1 February 2015; and for adult onset Pompe disease (AOPD) on 1 September 2015.

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

## 1. Disease prevalence

The prevalence of Pompe disease in Australia was fewer than 1 in 50,000 people and it is therefore suitable for the LSDP. The Panel noted that more people than expected are being diagnosed with Pompe disease each year. The Panel recommended that the Government should review the prevalence of Pompe disease in the next five years.

## 2. Treatment guidelines

The Panel noted that Pompe disease is referred to overseas as either infantile or late onset Pompe disease. Therefore, the Panel recommended that the LSDP update its wording to be the same.

The Panel also recommended that the LSDP review the medical tests required for paediatric and adult patients to be eligible for the program. This review needs to have input from treating physicians managing Pompe disease patients.

## 3. Medicine effectiveness and safety

People appear to live longer when receiving alglucosidase alfa (Myozyme®) for Pompe disease, compared to people not receiving treatment. This medicine 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:

- 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.

## 5. Value for money

People treated with alglucosidase alfa (Myozyme®) 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 this medicine 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 dose of alglucosidase alfa (Myozyme®) is based on the person's weight. It can be difficult to keep the dose accurate with young growing patients, so patients, their families/carers and healthcare staff should regularly tell the treating physician what the patient weighs. The Panel noted that it is easy and quick for the treating physician and the LSDP to change the dose whenever needed.

The Panel recommended that the drug company for alglucosidase alfa (Myozyme®) should 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 Panel noted that drug companies are developing new medicines for Pompe disease. There is one new medicine which could be ready for patients in the next two years.

## 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.