Australian Government
Department of Health and Aged Care

Life Saving Drugs Program (LSDP) Review of Medicines for Paroxysmal Nocturnal Haemoglobinuria (PNH) 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 PNH Review

At the time of the review, eculizumab (Soliris®) was the only medicine for PNH listed on the LSDP. It was listed on 1 January 2011.

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

1. Disease prevalence

The prevalence of PNH in Australia was fewer than one in 50,000 people and is therefore suitable for the LSDP. Because there have been more people diagnosed with PNH, the Panel recommended the prevalence of PNH be reviewed within five years.

2. Treatment guidelines

The Panel recommended that the LSDP Guidelines for PNH be updated so that eculizumab can be given every 12 to 16 days, instead of every 14 days. The Panel also noted that the drug company for eculizumab should give more information on how to use higher doses of the medicine. This would assist the LSDP in updating its Guidelines.

3. Medicine effectiveness and safety

The Panel noted that some people appear to live longer when receiving eculizumab, compared to people not receiving treatment.

PNH patients often require more than just eculizumab to manage their disease. The Panel noted that there have been improvements with overall PNH treatment which may contribute to PNH patients living longer. Overall, the Panel recommended that eculizumab remains suitable for the LSDP.

4. Treatment outcomes

The LSDP should 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 noted that the data the LSDP needs for PNH patients is similar to the information needed for complex medicine listings on the Pharmaceutical Benefits Scheme (PBS).

5. Value for money

People treated with eculizumab 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 eculizumab 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.

The Panel noted that eculizumab was also listed on the PBS for another disease. The Panel recommended that the Government look at whether this medicine should stay on the LSDP or move to the PBS.

6. Drug usage

The Panel noted that there have been more PNH patients than was expected when eculizumab was first put on the LSDP. The Panel therefore recommended that the Government review the pricing arrangement with the drug company.

7. Future treatments

The Panel noted that there was a new medicine available to treat PNH that could impact the LSDP. New treatments would likely affect the LSDP in the near future.

Post-Review update

In July 2021, another medicine to treat PNH, called ravulizumab (Ultomiris®), was approved to be added to the Pharmaceutical Benefits Scheme (PBS). A medicine cannot be on the LSDP when there is another medicine that treats the same disease on the PBS. Therefore, from 1 March 2022, eculizumab (Soliris®) moved from the LSDP to the PBS.

Because eculizumab (Soliris®) is no longer on the LSDP, the Panel's recommendations will not be actioned.