



# Life Saving Drugs Program (LSDP) 24-month Review Summary: Cerliponase alfa (Brineura<sup>®</sup>)

## Consumer Summary

### Introduction

Cerliponase alfa (brand name Brineura<sup>®</sup>) is a medicine used to treat Late Infantile Onset Batten Disease (CLN2 disease). It was listed on the LSDP on 1 May 2019. This review was considered by the LSDP Expert Panel (the Expert Panel) at its October 2021 meeting.

### Purpose of the 24-month review

All new medicines made available on the LSDP are reviewed after two years (24 months). The review aims to understand how the medicine had been used by patients in the 'real world' compared to what was expected at the time the medicine was first listed and what the cost of the medicine has been. The Terms of Reference (ToRs) for the cerliponase alfa (Brineura<sup>®</sup>) review covered the following issues:

1. **Medicine effectiveness and safety:** Have patients' tests results or symptoms improved from the medicine?
2. **Testing for treatment eligibility:** Are the tests results required by the LSDP to start the medicine still suitable? What about the tests required each year as part of the reapplication process?
3. **Medicine Usage:** Are the appropriate patients accessing the medicine? Are the LSDP Guidelines for the disease appropriate?
4. **Value for Money:** What is the cost of funding this medicine? Has this changed since the medicine was first available?

The LSDP Expert Panel (the Panel) is responsible for the reviews and making recommendations to the Chief Medical Officer (CMO). For more information on the LSDP and the review process, refer to the [LSDP](#) webpage.

### Review Outcomes – Recommendations

The Panel made the following recommendations in relation to cerliponase alfa:

1. That cerliponase alfa should remain on the LSDP as a treatment for CLN2 disease with no changes to eligibility criteria.
2. That the LSDP should make it easier for clinicians to provide the patient data required for applications. This will improve the everyday work of the program and future medicines reviews.

3. That, in consultation with clinicians, patient data collection should include quality of life, adverse events and survival data.
4. That the price that the Government pays for cerliponase alfa 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.

## **24-month review – summary of findings**

### **1 – Medicine effectiveness and safety**

The Panel noted that the Motor-Language (ML) scale was the most common way for doctors to monitor how patients were responding to cerliponase alfa (Brineura®) treatment. The ML score for treated patients took longer to decline compared to patients not on treatment. This means that patients receiving cerliponase alfa live longer than untreated patients. The Panel noted that there were often side effects from the medicine.

The Panel concluded that cerliponase alfa remains suitable for the LSDP. No changes were needed to the eligibility criteria.

### **2 – Testing for treatment eligibility**

There were no changes noted to how doctors diagnose CLN2 disease since 2018. There were also no increases in the number of people expected to be diagnosed with CLN2 disease. The Panel concluded that the LSDP criteria for who can receive cerliponase alfa remain suitable.

### **3 – Medicine usage**

The review found that there were fewer patients receiving the medicine than expected in the first year of cerliponase alfa being available on the LSDP.

The biggest cost for families was traveling to the hospital to receive the medicine and the time it took to receive it. However, patients' families said the benefit of receiving cerliponase alfa outweighed these costs.

ML score, survival and quality of life were considered very important outcomes for patients and their carers. Families described the changes in their children's quality of life as 'life saving'.

### **4 – Value for money**

The Expert Panel noted that the total costs to the Government for cerliponase alfa were lower than expected. It was recommended that the price that the Government pays for cerliponase alfa 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.

## **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.