Australian Government
Department of Health and Aged Care

Life Saving Drugs Program (LSDP) 24 month-like Review Summary: elosulfase alfa (Vimizim®)

Consumer Summary

Introduction

Vimizim® (elosulfase alfa) is a medicine used to treat Mucopolysaccharidosis Type IVA (MPS IVA), also known as Morquio A syndrome. It was listed on the LSDP on 1 August 2017. The LSDP Expert Panel (Expert Panel) agreed at its October 2021 meeting that elosulfase alfa should be reviewed when its current pricing agreement ended. This review was considered by the Expert Panel at its October 2022 and February 2023 meetings.

Purpose of the 24 month-like review

The review aims to understand how the medicine has 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 elosulfase alfa review covered the following issues:

1. **Clinical effectiveness and safety**: Have patients' tests results or symptoms improved after using the medicine?
2. **Test Validity and Usage**: 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. **Utilisation and Consumer Impact**: Are the appropriate patients accessing the medicine? Are the LSDP Guidelines for the disease appropriate?
4. **Financial Impact**: What is the cost of funding this medicine? Has this changed since the medicine was first available?

The Expert Panel is responsible for the reviews and making recommendations to the Chief Medical Officer. For more information on the LSDP and the review process, please refer to the LSDP webpage.

Review Outcomes – Recommendations

The Expert Panel made the following recommendations in relation to elosulfase alfa:

1. That elosulfase alfa should remain on the LSDP as treatment for MPS IVA.
2. That the tests patients need to start treatment (initial eligibility criteria) and to continue treatment each year (ongoing eligibility criteria) should be changed:
    a. The requirement for specific breathing and heart tests should be removed.

b. To prove that a patient is eligible to start treatment, a biochemical urine test will be needed to show that they have the disease. The test will need to be done again after the patient has started treatment to show that the medicine has started working. This is explained further in the Summary of Findings below.
c. That the ongoing eligibility requirements should be updated:
    I. Only a clinic letter will be required, but this should include the results of any tests the treating physician feels are appropriate to monitor a patient who has this disease.
    II. Patients can miss infusions and still be eligible for ongoing treatment.
    III. For patients whose urine test did not show that they are responding to treatment, the treating physician must write a clinic letter to the LSDP explaining that the patient is doing well on the medicine and include any extra tests and information for the LSDP to review.

3. That the price that the Government pays for elosulfase alfa should 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.

Summary of Review findings

1 – Medicine effectiveness and safety

Treating physician and consumer consultation suggested that the tests the LSDP needed to show continued eligibility for the medicine did not measure all aspects of a patient's response to treatment. The Expert Panel noted that for LSDP patients, there was large variation in some test results, possibly due to the young age of the patients. The current eligibility test requirements seemed to have limited value for assessing the effectiveness of treatment.

The Expert Panel noted that there were often side effects from the medicine and although these were frequent, treating physicians and patients reported they were usually easy to manage.

The Expert Panel concluded that elosulfase alfa remains suitable for the LSDP. Changes were identified for the initial and ongoing eligibility criteria.

2 – Testing for treatment eligibility

The diagnosis of MPS IVA can be challenging, and delays in diagnosis are common. The Expert Panel recommended that the biochemical urine test will be required 2 to 3 different times to establish initial eligibility, including:

i. at diagnosis (to establish a starting point for each patient)
ii. 1 to 3 months after the medicine is started
iii. if a 20% drop is not shown at (ii) – 6 months after the medicine is started.

3 – Medicine usage

The number of patients receiving elosulfase alfa on the LSDP at the time of the review was as expected at the time the medicine was added to the program.

Families of patients receiving elosulfase alfa on the LSDP make large financial and lifestyle sacrifices to access treatment. However, overall patients reported a positive experience in receiving their weekly treatment, and families believed sacrifices were outweighed by the benefits patients experienced. Quality of life related impacts mattered most to families, rather than the specific clinical tests monitored by treating physicians and reported to the LSDP.

4 – Value for money

The Expert Panel noted the total cost to Government for elosulfase alfa is currently high in comparison to other enzyme replacement therapies on the LSDP due to the high price per patient. It was recommended the price that the Government pays for elosulfase 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 Chief Medical Officer has agreed to these recommendations. The implementation of these recommendations is currently being considered and progressed by the Department of Health and Aged Care in consultation with the drug company, treating physicians, and patient advocacy groups.