Australian Government
Department of Health and Aged Care

Life Saving Drugs Program (LSDP)

Eliglustat (Cerdelga®) outcome statement

Overview:

The LSDP Expert Panel (the Expert Panel) advises the Chief Medical Officer (CMO) on new medicine applications to the LSDP. Eliglustat was re-considered for listing on the LSDP at the Expert Panel's meetings on 18 February 2022, 24 June 2022 and out of session, and the CMO recommended eliglustat for listing on the LSDP in December 2022.

Background:

Eliglustat is approved by the Therapeutic Goods Administration (TGA) for use in patients with Gaucher disease (type 1). Gaucher disease (type 1) is an inherited disorder, caused by mutations in the GBA gene. The GBA gene codes for the enzyme beta-glucocerebrosidase, which is used to break down the fatty substance glucocerebroside into glucose and a simpler fat molecule, ceramide. Tissues and organs are damaged by the abnormal accumulation and storage of these substances, causing the characteristic features of Gaucher disease – abnormalities in the bones, liver and blood, and lung disease.

Pharmaceutical Benefits Advisory Committee (PBAC) Consideration:

At its July 2015 meeting, PBAC considered a submission from Sanofi requesting the listing of eliglustat on the Pharmaceutical Benefits Scheme (PBS) for the treatment of patients with Gaucher disease (type 1). PBAC did not recommend the requested listing of eliglustat on the basis that 'clinically important inferiority could not be excluded with confidence'. PBAC noted the submission's request that consideration be given to including eliglustat on the LSDP and further noted that, at that time, the CMO was responsible for advising the Minister on drugs proposed for inclusion on the LSDP.

Further information on PBAC's consideration of eliglustat including reasons for rejection is available in the PBAC Public Summary Document: www.pbs.gov.au/pbs/industry/listing/elements/pbac-meetings/psd/2015-07/eliglustat-psd-july-2015.

Previous CMO Consideration:

In July 2017, prior to the establishment of the Expert Panel, the CMO recommended the listing of eliglustat on the LSDP in a strict second line setting for Gaucher disease (type 1). Negotiations ended when the Department and the sponsor were not able to agree on the place in therapy for eliglustat.

Expert Panel Consideration:

The Expert Panel was established in June 2018. At the request of the sponsor, the Expert Panel reconsidered the application for eliglustat in November 2019. The Expert Panel deferred its decision until the Government-agreed outcomes from the Gaucher disease LSDP medicines review were known. Following completion of the Gaucher disease review, the Expert Panel reconsidered eliglustat at its February 2022 and June 2022 meetings and out of session. The Expert Panel accepted the CMO's previous position regarding eliglustat's eligibility for the LSDP. Rather than a strict second line setting, the Expert Panel agreed that Gaucher disease (type 1) patients could access eliglustat after 12 months of treatment with enzyme replacement therapy. In December 2022 the sponsor and the Department agreed on a price for eliglustat based on the Expert Panel's recommendations.

Pricing issues:

Note that the price of all LSDP medicines are subject to commercial in confidence arrangements.

Consumer Input:

The Expert Panel noted the stakeholder input from the LSDP application. The Expert Panel found the insight provided by the stakeholders was informative and contributed to its deliberations.

Treatment Guidelines:

The diagnosis of Gaucher disease (type 1) must be established by the demonstration of specific deficiency of glucocerebrosidase enzyme activity in leukocytes or cultured skin fibroblasts, or by the presence of mutations in the glucocerebrosidase gene, known to result in severe deficiency of enzyme activity, in tissue or peripheral blood leukocytes.

The patient must satisfy at least one of the following criteria to be eligible for treatment with eliglustat:

Symptomatic Gaucher disease (type 1) with any of the disease manifestations listed below:

a. Skeletal: Evidence of skeletal disease beyond mild osteopenia or Erlenmeyer flask deformity, as assessed by symptoms, skeletal survey and MRI.
b. Haematological complications: Haemoglobin <105g/L for females and < 115g/L for males (at least 2 measurements more than 1 month apart and having excluded other causes, e.g. iron deficiency); or platelet count < 120 $x10^9$/L on at least 2 occasions (more than 1 month apart).
c. Gastrointestinal complications: liver volume (CT or MRI) ≥1.25 x normal; or spleen volume (CT or MRI) > 5x normal. Patients under 16 years of age with symptomatic Gaucher disease with any relevant physical signs may be treated prior to confirmation of the type of Gaucher disease. Formal ophthalmologic review and neurodevelopmental status reports should be provided with the application.

Additional eligibility requirements for access to subsidised eliglustat:

a. Patients must have had ERT for at least 12 months before commencing eliglustat; or be intolerant to ERT; or be unable to receive intravenous infusions; and
b. patients must be 18 years or older.

Management of Uncertainties:

To address uncertainties, clinical data will be collected through initial and ongoing applications to the LSDP. In line with LSDP policy and to manage uncertainties, a review of eliglustat will be conducted 24 months after listing to ensure use and performance of the medicine is in line with the expectations at the time of listing.

Context:

The LSDP provides access for eligible patients with rare and life-threatening diseases to essential and very expensive medicines. The LSDP provides eligible patients with access to these life-saving medicines at no expense to the patients or their families.

Before being considered for inclusion on the LSDP, a drug must first be considered by PBAC and accepted as clinically effective but rejected for PBS listing because it fails to meet the required cost effectiveness criteria.

All applications for new medicines seeking funding through the LSDP are considered by the Expert Panel. The role of the Expert Panel is to provide advice and assistance to the CMO on a range of matters relating to new medicines seeking funding, including assessment of how the medicine addresses the LSDP criteria, guidelines for medicine use and testing requirements, suitable pricing arrangements, and data collection required for future reviews.

After receiving advice from the Expert Panel, the CMO advises the Minister for Health and Aged Care on medicines proposed to be included on the LSDP.

This document aims to provide an overview of the evidence considered by the Expert Panel and CMO during their assessment of medicines.

For more information on the process for new medicines seeking funding through the LSDP, refer to the LSDP Procedure guidance: www.health.gov.au/resources/publications/procedure-guidance-for-medicines-funded-through-the-life-saving-drugs-program-lsdp

Sponsor's Comment:

Sanofi welcomes the LSDP Expert Panel's advice to the CMO, and the CMO's recommendation to list Cerdelga (eliglustat) on the LSDP. Cerdelga provides an oral option for people living with Gaucher disease (type 1).