21 April 2017
EMA/CHMP/208415/2017
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Brineura

cerliponase alfa

On 21 April 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Brineura, intended for the treatment of CLN2 disease. Brineura, which was designated as an orphan medicinal product on 12 March 2013, was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is BioMarin International Limited.

Brineura will be available as a solution for intracerebroventricular infusion (150 mg). The active substance of Brineura is cerliponase alfa, an enzyme replacement therapy (ATC code: A16AB). It works by replacing the missing enzyme tripeptidyl peptidase-1 in patients with a lysosomal storage disorder called CLN2 disease (neuronal ceroid lipofuscinosis type 2), reducing build-up of lipofuscins.

The benefits with Brineura are its ability to slow the progression of motor and language decline. The most common side effects are pyrexia, vomiting and hypersensitivity.

The full indication is: "Brineura is indicated for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase 1 (TPP1) deficiency."

Brineura must only be administered by a trained healthcare professional knowledgeable in intracerebroventricular administration.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
Send a question via our website www.ema.europa.eu/contact