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Media and Public Relations

**Press release**

# New medicine for rare neurodegenerative disorder in children

## Brineura reviewed under accelerated assessment

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for Brineura (cerliponase alfa) for the treatment of a very rare, fatal neurodegenerative condition in children called neuronal ceroid lipofuscinosis type 2 (CLN2) disease.

Children with this disease are unable to produce enough of the enzyme tripeptidyl-peptidase 1, which plays a role in breaking down certain proteins inside the cells. This causes the build-up of protein deposits in the cells, including nerve cells, which damage tissues and lead to progressive degeneration of the brain and retina. Most children affected lose their ability to walk and talk by the age of six. The disease usually leads to the death of the child between the ages of eight and 12.

There are currently no medicines approved for treatment of CLN2 disease. Current options only treat the symptoms of the disease.

Brineura, which contains a recombinant form of tripeptidyl-peptidase 1, is designed to replace the missing enzyme, and is thereby expected to improve some of the symptoms experienced by the young patients.

The safety and efficacy of Brineura were assessed in a single-arm, open-label phase I/II study involving 24 children aged between three and eight years and an extension of this study which evaluated the long-term effects of the medicine.

The study also compared the improvement of motor and language functions in children treated with Brineura with information from natural history studies that observe how the disease develops in untreated children over time.

In the study, 20 of the 23 patients treated (and who could take part in the efficacy analyses) experienced either a slower than expected progression of the disease, a stabilisation of the progression of the disease or some improvement in their motor and language abilities. This was considered a significant therapeutic effect.

In the extension study, the slowdown in the progression of the disease was observed for more than one year and occurred even when the disease was already advanced.

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The most frequent adverse events were fever, vomiting, hypersensitivity, seizures and upper respiratory tract infections.

As CLN2 is a very rare disease, the Committee for Medicinal Products for Human Use (CHMP) agreed that it is not possible to provide comprehensive data on the efficacy and safety under normal conditions of use. Therefore the Committee recommended granting a marketing authorisation under exceptional circumstances and requested the applicant to complete an ongoing study that is part of the paediatric investigation plan to further evaluate the safety, efficacy and tolerability of Brineura, with a particular focus on children younger than two. The company will also monitor the long term safety of the medicine through a patient registry.

CLN2 is a very rare disease and patients with the condition have a significant unmet medical l need. The Agency has a number of mechanisms to encourage the development of medicines in such situations.

Brineura received an orphan designation from EMA's Committee for Orphan Medicinal Products (COMP) in 2013, with the consequent incentives including free scientific advice on the clinical and non-clinical aspects of the medicine's dossier. Once the application was made, the Agency reviewed it under its accelerated assessment programme, designed to facilitate access to medicines that meet an unmet medical need.

The company also received advice from EMA's Paediatric Committee (PDCO) on its development programme.

The use of the authorisation under exceptional circumstances route allows patients access to medicines that cannot be approved under a standard authorisation as comprehensive data cannot be obtained, either because there are only very few patients with the disease, the collection of complete information on the efficacy and safety of the medicine would be unethical, or there are gaps in the scientific knowledge. These medicines are subject to specific post-authorisation obligations and monitoring.

The opinion adopted by the CHMP at its April 2017 meeting is an intermediary step on Brineura's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, a decision on price and reimbursement will then take place at the level of each Member State considering the potential role/use of the medicine in the context of the national health system of that country.

## Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Brineura is BioMarin International Limited.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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