



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Press Office

## Press release

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# Cerdelga recommended for approval in type 1 Gaucher disease

## New oral treatment option for rare debilitating genetic disease

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for Cerdelga (eliglustat), for the treatment of type 1 Gaucher disease, a rare, debilitating and life-threatening genetic disease.

Gaucher disease is estimated to affect approximately 15,000 people in the EU. It is caused by the lack of an enzyme which normally breaks down a fatty waste product called glucosylceramide (also called glucocerebroside). Without the enzyme, glucosylceramide builds up in the body, typically in the liver, spleen and bone marrow. This causes a wide range of symptoms, including anaemia (low red blood cell counts), tiredness, easy bruising and a tendency to bleed, an enlarged spleen and liver, and bone pain and fractures.

Treatment options for Gaucher disease in the EU are currently limited. Patients can take Cerdelga orally, which could provide a convenient alternative to the most often used treatments given by intravenous injection. The medicine acts by preventing excessive increase of the levels of glucosylceramide in the body. In phase III clinical trials conducted in over 200 patients, Cerdelga has shown a convincing efficacy with an acceptable safety profile.

Cerdelga was designated as an orphan medicine and EMA provided protocol assistance to the applicant during the development of the medicine. Orphan designation and the associated incentives such as free scientific advice or protocol assistance are among the Agency's most important instruments to encourage the development of medicines for patients suffering from rare diseases.

The opinion adopted by the Committee for Medicinal for Human Use (CHMP) at its November 2014 meeting is an intermediary step on Cerdelga'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, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.



## Notes

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1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Cerdelga is Genzyme Europe BV.
3. More information on the work of the European Medicines Agency can be found on its website:  
[www.ema.europa.eu](http://www.ema.europa.eu)

## Contact our press officer

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