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# EC grants marketing authorization for Cerdelga

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Gaucher disease is an inherited condition affecting fewer than 10,000 people worldwide.

A small number of adult patients who metabolize Cerdelga more quickly or at an undetermined rate, as detected by an established genetic laboratory test, will not be eligible for Cerdelga treatment.

Cerdelga was approved by the USFDA in August 2014, and is under review by other regulatory authorities around the world.

It is expected that Cerdelga will be available commercially in EU countries beginning in 2015 and over the next few years.

Cerdelga is a potent, highly specific ceramide analogue inhibitor of glucosylceramide synthase with broad tissue distribution including to bone marrow.

It reduces the production of glucosylceramide, the substance that builds up in the cells and tissues of people with Gaucher disease type 1.

Cerdelga is indicated in the European Union for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolizers (PMs), intermediate metabolizers (IMs) or extensive metabolizers (EMs).

The majority of adverse reactions of Cerdelga are mild and transient. The most commonly reported adverse reaction with Cerdelga is diarrhea, in approximately 6 percent of the patients.

The incidence of diarrhea was the same or higher with placebo than with Cerdelga in the placebo-controlled pivotal study. Less than 2 percent of patients receiving Cerdelga permanently discontinued treatment due to any adverse reaction.

The EC approval was based on data from the Cerdelga clinical development program, the largest clinical research program ever conducted in Gaucher disease type 1, with approximately 400 patients treated in 29 countries.