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Genzyme, a Sanofi company, announced that the Ministry of Health, Labour and Welfare (MHLW) has granted marketing approval for Cerdelga, which is claimed to be the only oral therapy for the treatment of adults with Gaucher disease type 1 in Japan. Cerdelga was approved by the U.S. Food and Drug Administration in August 2014, the European Commission in January 2015, and the Australian Therapeutics Goods Administration in February 2015.

The MHLW 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.

The development program included three Phase 3 clinical trials. In the Phase 3 treatment-naÃ?Æ?Ã?Â¨ve, placebo-controlled trial, ENGAGE, improvements were seen across the following endpoints after 9 months on Cerdelga: spleen size, platelet levels, hemoglobin levels, and liver volume.

The second Phase 3 trial, ENCORE, was designed to assess disease stability in patients previously treated with enzyme replacement therapy. That trial met the pre-specified criteria for non-inferiority to an enzyme replacement therapy (imiglucerase), which was a composite endpoint of the following parameters: spleen volume, hemoglobin levels, platelet counts, and liver volume.

The third Phase 3 trial, EDGE, included 10 Japanese patients, and was designed to evaluate different dosing frequencies of Cerdelga (in patients previously treated with enzyme replacement therapy or treatment naÃ?Æ?Ã?Â¨ve Gaucher disease patients).

The EDGE trial included an interim analysis of efficacy and safety in Japanese patients. Patients in all three Phase 3 studies continue to receive Cerdelga in the extension periods, and the majority of patients are in their 4th or 5th year of treatment.

Gaucher disease is an inherited condition affecting fewer than 10,000 people worldwide. People with Gaucher disease do not have enough of an enzyme, acid Ã?Å½Ã?Â²-glucosidase (glucocerebrosidase) that breaks down a certain type of fat molecule. As a result, lipid engorged cells (called Gaucher cells) amass in different parts of the body, primarily the spleen, liver and bone marrow.

Accumulation of Gaucher cells may cause spleen and liver enlargement, anemia, excessive bleeding and bruising, bone disease and a number of other signs and symptoms. The most common form of Gaucher disease, type 1, generally does not affect the brain.