News Releases

Sanofi Genzyme Announces Approval of CerdelgaTM (eliglustat capsules) by Health Canada for Rare Genetic Condition

Mississauga, ON – May 1, 2017 – Sanofi Genzyme, a division of Sanofi-Aventis Canada Inc., announced today that Health Canada has recently approved Cerdelga™ (eliglustat capsules), the only first-line oral therapy indicated for the treatment of adult patients with Gaucher Disease Type 1.[1] A rare genetic metabolic condition that causes fats to build up in certain parts of the body, Gaucher Disease Type 1 is progressive and may become life-threatening if symptoms are left untreated.[2,3] Patients who are specific enzyme metabolisers, as detected by a simple test, will be eligible for Cerdelga treatment.[1]

“Patient experience with Cerdelga indicates that this new treatment has a beneficial effect on a number of symptoms associated with Gaucher Disease Type 1 and significantly improves quality-of-life due to the elimination of burdensome bi-weekly intravenous infusions,” said Christine White, President of the National Gaucher Foundation of Canada. “We are very excited that this new first-line oral therapy is now available in Canada for people living with Gaucher Disease Type 1.”

How Cerdelga works
Current therapies for Gaucher Disease Type 1, either enzyme replacement therapy (ERT) or substrate reduction therapy (SRT), aim to reduce the amount of glucosylceramide (GL-1), a fatty substance found in cells, and diminish the progression of symptoms which can damage the spleen, liver and bones.[2] Cerdelga, a SRT, decreases production of GL-1 by blocking the absorption of materials that are used by cells to make GL-1, thereby minimizing the effects of the disease.[4,5]

Efficacy and safety
Cerdelga has been evaluated in a clinical program for Gaucher Disease involving almost 400 patients (including 11 Canadians) and two Phase 3 clinical studies.[6,7,8,9,10] In untreated patients with Gaucher Disease Type 1, Cerdelga produced improvements in platelet and hemoglobin levels, spleen and liver volumes, and bone outcomes.[11,7] In patients whose disease had been stabilized with ERT, Cerdelga maintained stability of these disease parameters.[6]

In an integrated safety analysis of data from all clinical trials to date, most adverse events reported were mild to moderate in severity.[10]

“The approval of an oral therapy for Gaucher Disease is a welcome addition to patients,” said Dr. Aneal Khan, metabolic physician, Department of Medical Genetics, Alberta Children’s Hospital, Calgary, AB. “Having an oral therapy that is well tolerated and works on the various aspects of Gaucher Disease will make therapy easier. However, it is important that we still have ERT as a suitable option for patients.”

Sanofi Genzyme’s legacy with Gaucher Disease
Sanofi Genzyme has been researching an oral therapy for Gaucher Disease for fifteen years. The Cerdelga clinical development program is the largest ever conducted in Gaucher Disease, with almost 400 patients treated in 29 countries, including Canada.[6,7,8,9,10]

“The approval of Cerdelga represents a significant milestone in the journey that Sanofi Genzyme initiated years ago with the Canadian Gaucher Disease community,” said Peter Brenders, General Manager, Sanofi Genzyme Canada. “Our dedication, commitment and collaboration with this patient population continues to transform lives and inspires us to work towards our long term goal of continuous innovative research and empowering Gaucher patients.”

About Gaucher Disease Type 1
Gaucher Disease (pronounced go-shay) is a rare genetic metabolic condition caused by deficient activity of the enzyme glucocerebrosidase.[2] The function of this enzyme is to break down waste material within cells.[2] The waste material that builds up is a fatty substance called glucosylceramide (GL-1), which can lead to a spectrum of potentially life-threatening symptoms.[2] These symptoms can vary widely among individuals and may include progressive spleen and liver enlargement, thrombocytopenia (low blood platelets), bone pain and fractures, and ultimately impaired spleen and bone function.[12,13]

Estimated to affect from 1 in 40,000 to 1 in 60,000 individuals, Type 1 is the most common form, and is especially prevalent among those with Ashkenazi Jewish ancestry, occurring in approximately 1 in 855 individuals.[14,15,12,2] In Canada alone, Type 1 affects approximately 150 people.[16]

The full Product Monograph for Canada is available here.

Sanofi Genzyme is committed to helping patients who are prescribed Cerdelga gain access to the medicine and receive the support they may need. It will offer a patient support program called LIBERTY which will provide follow up service calls, as well as reimbursement assistance service. For more information, please email info@LibertyPSP.ca.

About Sanofi
Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi is organized into five global business units: Diabetes and Cardiovascular, General Medicines and Emerging Markets, Sanofi Genzyme, Sanofi Pasteur and Sanofi Consumer Healthcare. Sanofi is listed in Paris (EURONEXT: SAN) and in New York (NYSE: SNY).

In Canada, we employ close to 1,900 people. In 2015 Sanofi companies invested $133.3 million in R&D in Canada, creating jobs, business and opportunity throughout the country.

Sanofi Genzyme focuses on developing specialty treatments for debilitating diseases that are often difficult to diagnose and treat, providing hope to patients and their families. Learn more at www.sanofigenzyme.ca.

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