Sanofi announces new safety data for investigational avalglucosidase alfa in patients with Pompe disease

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Results from the Phase 1/2 trial (NEO1) and its ongoing extension study (NEO-EXT) show consistent safety data for Sanofi’s investigational avalglucosidase alfa (neoGAA) in patients with late-onset Pompe disease (LOPD) dosed over 3.5 years, without evidence of inhibition of enzyme activity over the analysis period. The study results were presented today at the 14th Annual WORLDSymposium 2018 in San Diego, California.

The NEO1 study enrolled 24 LOPD patients, including 10 patients who had not previously been on treatment and 14 who had been treated with alglucosidase alfa for at least nine months. After completing NEO1, in which patients received avalglucosidase alfa at 5, 10 or 20 mg/kg once every other week, 19 of 24 patients entered the NEO-EXT study, of which 18 are still enrolled. All patients in the NEO-EXT study have switched to receive avalglucosidase alfa 20 mg/kg once every other week. Participants who remain in the study continue receiving avalglucosidase alfa for a planned 6-year follow-up.

Treatment-emergent adverse events (AEs) were mostly mild across both studies at all dose levels. The most common AEs were nausea, headache and fatigue, each occurring in 3/24 patients, and dizziness, redness of the skin (erythema), muscle spasm, muscle pain (myalgia), shortness of breath (dyspnea) and rash, each occurring in 2/24 patients. No deaths or life-threatening AEs have been reported. In addition, no patient developed immunoglobulin E antibodies or tested positive for inhibition of enzyme activity during the analysis period of the NEO1 study. One participant discontinued from NEO1 due to a serious AE and infusion-associated reaction of respiratory distress and chest discomfort. One person has discontinued NEO-EXT for personal reasons.

“These results are reassuring and indicate that the safety data for avalglucosidase alfa is consistent in longer term treatment with what we saw in the initial six months of the trial,” said Loren D.M. Pena, M.D., Ph.D, Associate Professor of Pediatrics at Duke University School of Medicine. “These data add to the body of research supporting avalglucosidase alfa as a potential new treatment option for patients living with Pompe disease.”

“The development of avalglucosidase alfa builds on Sanofi’s decades of commitment to research targeting new treatments for unmet needs in rare diseases, including Pompe disease,” said Rand Sutherland, M.D., Global Head, Rare Disease Development for Sanofi. “These results provide important additional momentum to our efforts to potentially bring avalglucosidase alfa to patients with Pompe disease.”

Pompe Disease

Pompe disease is a progressive, debilitating and often fatal neuromuscular disease caused by a genetic deficiency or dysfunction of the lysosomal enzyme acid alpha-glucosidase (GAA) that results in the buildup of glycogen in the body’s cells. The disease affects an estimated 50,000 people worldwide and can occur at any age from infancy to late adulthood.

Pompe disease is often classified as late-onset (LOPD) or infantile-onset Pompe disease (IOPD). Patients with LOPD typically present any time after the first year of life to late adulthood. The hallmark symptom of LOPD is skeletal muscle weakness, which often leads to walking disability and reduced respiratory function. Patients often require wheelchairs to assist with mobility and may require mechanical ventilation to help with breathing. Pompe disease is classified as IOPD when symptoms begin within a few months of birth and there is impact to the heart in addition to skeletal muscle weakness.

Avalglucosidase Alfa Clinical Development Program

Avalglucosidase alfa is an investigational enzyme replacement therapy that has been designed for selective receptor targeting and enzyme uptake, with the aim of enhancing glycogen clearance.

In addition to the NEO-EXT trial, avalglucosidase alfa is being evaluated in the COMET trial, a currently recruiting Phase 3 study to compare the efficacy and safety of bi-weekly infusions of avalglucosidase alfa and alglucosidase alfa in patients with LOPD who have not previously been on treatment. The investigational therapy is also being studied in pediatric patients with IOPD previously treated with alglucosidase alfa. For more information on these trials, please visit https://www.clinicaltrials.gov/.
Avalglucosidase alfa is an investigational agent and has not been approved by the US Food and Drug Administration (FDA) or any other regulatory agency worldwide for the use under investigation.

**About Sanofi**

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

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