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Genzyme, a Sanofi company, has announced that the US Food and Drug Administration (US FDA) has granted Breakthrough Therapy designation to olipudase alfa. This enzyme replacement therapy is being investigated for the treatment of patients with non-neurological manifestations of acid sphingomyelinase deficiency (ASMD), also known as Niemann-Pick disease type B, as opposed to type A which is characterized by neurological involvement.

ASMD is a serious and life-threatening disorder caused by insufficient activity of the enzyme acid sphingomyelinase (ASM), which results in the toxic accumulation of sphingomyelin. There are currently no approved treatment options for patients with Niemann-Pick disease type B.

Breakthrough Therapy designation is intended to expedite the development and review of investigational new drugs that target serious or life-threatening conditions. The criteria for granting Breakthrough Therapy designation are preliminary clinical evidence of substantial improvement on a clinically significant endpoint over available therapies. The Breakthrough Therapy designation is distinct from the FDA's other mechanisms to expedite drug development and review, and will allow for a close collaboration between Genzyme and the FDA on the olipudase alfa development program.

Olipudase alfa is being developed by Genzyme to potentially address the fundamental defect underlying the disease. Supplementing the defective or deficient native enzyme with olipudase alfa allows for the breakdown of sphingomyelin, whose accumulation is responsible for the clinical manifestation of ASMD.

The Breakthrough Therapy designation is supported by data from a completed Phase 1b study of olipudase alfa. Findings in five adult patients with nonneuronopathic ASMD were presented at the Lysosomal Disease Network's WORLD Symposium in February 2015. The data presented on the repeat-dose safety, pharmacodynamics, and exploratory efficacy of olipudase alfa support its continued development for the investigational use in nonneurological manifestations of ASMD.

The company has started enrollment of a Phase 1/2 pediatric study and is preparing for enrollment of a Phase 2/3 adult study in the second half of 2015.

"There is tremendous unmet need in the ASMD/Niemann-Pick disease type B community, and we are hopeful that olipudase alfa can be developed into a meaningful treatment for patients. We appreciate the FDA's support for this important program giving us the opportunity to utilize an important expedited drug development pathway for olipudase alfa and providing hope for patients affected with a chronic and progressively debilitating disease," said Dr Richard Peters, Genzyme's Global Head of Rare Diseases.