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Researchers report on promising new therapy for devastating genetic disorder

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A promising new therapy has – for the first time – reduced damage to the brain that can be caused by Sanfilippo B (MPS IIIB), a rare and devastating genetic disease, Los Angeles Biomedical Research Institute (LA BioMed) researchers will report today in a presentation at the Lysosomal Disease Network's 10th Annual WORLD Symposium.

Sanfilippo B syndrome is a lysosomal storage disease belonging to the group of mucopolysaccharidoses (MPS) that is characterized by severe and rapid intellectual deterioration. LA BioMed pioneered the research that led to the first enzyme replacement therapy for MPS I, Aldurazyme©, which has saved hundreds of lives in the nearly 11 years since it was approved by the Food and Drug Administration.

LA BioMed's MPS Research Laboratory reported the results of its latest study for treating Sanfilippo B syndrome in a disease model that employed recombinant human "NAGLU-IGF2" (a novel fusion protein of alpha-N-acetyglucosaminidase, or NAGLU, coupled to a peptide derived from the insulin-like growth factor, IGF2). The fusion protein was produced by BioMarin Pharmaceutical Inc.

LA BioMed researcher Shih-hsin Kan reported that the laboratory's tests found NAGLU-IGF2 is taken up by neurons in the disease model, which then reduces brain damage by reversing heparan sulfate storage – one of the causes of damage to the brain in patients with Sanfilippo B syndrome. The researchers concluded that NAGLU-IGF2 can be the basis for enzyme replacement therapy for Sanfilippo B syndrome.

"This is an exciting new development that needs to be examined further to determine its effectiveness in patients with Sanfilippo B syndrome who currently have no therapies available to reduce the damage to their brains," said Patricia Dickson, MD, director of the MPS Research Laboratory at LA BioMed. "We look forward to continuing our partnership with BioMarin in developing therapies for MPS."

BioMarin announced Tuesday that it has developed BMN 250, a manufactured form of NAGLU-IGF2, for the treatment of Sanfilippo B syndrome and expects to initiate clinical studies with BMN 250 in mid-2015.

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