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Researchers Surprised by Similar Structures in Sanfilippo Syndrome and Alzheimer's Disease

Rare Genetic Disorder May Benefit from Development of New Dementia Treatments

LOS ANGELES – (May 4, 2009) – Researchers seeking to understand the causes of a rare genetic lysosomal storage disease, Sanfilippo syndrome type B, were surprised to find protein aggregates, known as neurofibrillary tangles, that are usually seen in Alzheimer's and other forms of dementia, according to a study published May 4 in the Proceedings of the National Academy of Sciences.

The discovery, in a study conducted at the Los Angeles Biomedical Research Institute (LA BioMed) and the University of California, Los Angeles (UCLA), means that the childhood dementia often seen in lysosomal storage diseases may have mechanisms similar to those found in Alzheimer's disease and other age-related dementias, which are characterized by an abnormal accumulation of the protein, P-tau.

The scientists also said these findings mean those suffering from these rare disorders could one day benefit from the abundance of research underway for the growing numbers of Alzheimer's patients.

"We were trying to determine what in the cellular environment makes certain brain regions more vulnerable to neurodegeneration, and we were surprised to find the presence of a protein, P-tau, and the corresponding aggregates similar to neurofibrillary tangles we see in Alzheimer's patients," said Stanislav L. Karsten, PhD, a corresponding author of the study and a LA BioMed investigator. "With the increasing incidence of Alzheimer's disease, we expect to see new drugs developed to prevent these neurofibrillary tangles or dissolve them. Our findings suggest those treatments could also benefit patients with this rare genetic disorder, Sanfilippo syndrome."

Sanfilippo syndrome, also known as MPS III, occurs in approximately one in 70,000 births, causing profound mental retardation, behavior problems and, frequently, death before the age of 20. It is one of nearly 40 lysosomal storage diseases caused by the body's inability to produce enzymes that break down and recycle materials in cells. Without the enzymes, materials are stored in virtually every cell of the body, causing severe damage over time. There is no treatment for Sanfilippo syndrome.

Alzheimer's disease affects 2 million to 4 million Americans, and their ranks are expected to grow to as many as 14 million by the middle of the 21st century as the population ages.

Grants from the National Institutes of Health, the Children's Medical Research Foundation, the National Alliance for Research on Schizophrenia and Depression and the Alzheimer's Association funded this research.

For copies of the study, "Sanfilippo syndrome type B, a lysosomal storage disease, is also a tauopathy," please contact Laura Mecoy, 310.546.5860, or [LMecoy@issuesmanagement.com](mailto:Lmecoy@issuesmanagement.com)

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Founded 56 years ago, LA BioMed at Harbor-UCLA Medical Center is one of the country's largest nonprofit independent biomedical research institutes. It conducts biomedical research, trains young scientists and provides community services, including childhood immunization and nutrition assistance. The institute's researchers conduct studies in such areas as cardiovascular disease, emerging infections, cancer, diabetes, kidney disease, dermatology, reproductive health, vaccine development, respiratory disorders, inherited illnesses and neonatology. LA BioMed is academically affiliated with the David Geffen School of Medicine at UCLA. Please visit our website at www.LABioMed.org

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