

**Dr. Lluís Samaranch Gusi, University of California –**

AAV9-mediated human acid sphingomyelinase expression in nonhuman primate brain:  
Preclinical development of gene therapy for Niemann-Pick disease type A

**Layman Summary –**

The goal of this project is to develop a treatment for the neurological consequences of Niemann-Pick Type A Disease. In this project, we will evaluate the feasibility of delivering an adeno-associated virus encoding human acid sphingomyelinase globally into the brain by injection of a vector into the brain and/or the spinal canal. The Bankiewicz laboratory has over 30 years of experience in brain delivery in primates with a strong record in translational programs. Development of a treatment for Niemann-Pick type A disease has been supported over the last few years by an National Institutes of Health and National Institute of Neurological Disorders and Stroke RO1 grant to Dr. Bankiewicz. Since Dr. Gusi joined the lab, he has been working on delivery of AAV9 (adeno-associated virus 9) vectors into the brain and infusion of AAV2-hASM (adeno-associated virus 2- human acid sphingomyelinase).