Intracranial AAV gene therapy for Tay-Sachs and Sandhoff diseases

	Activities completed	Funding agencies
2007-2012	. Therapeutic efficacy shown in SD mice and cats . Therapeutic benefit in TSD sheep . Pre-IND meeting with FDA . IND-enabling tox studies completed in mice	NTSAD CTSF NIH
2012	. Final safety study in normal non-human primates shows severe toxicity of original AAV vectors	NTSAD CTSF
2014-2015	. Development of new AAV vector . Safety testing in NHP . Preliminary efficacy testing in SD mice . FDA recommendation for IND-enabling studies	
2016-2017	. Completed *in vivo* phase of IND-enabling safety and efficacy studies in SD mice . Completed *in vivo* phase IND-enabling safety study in normal NHPs	
2018	. Analyses completed and met all pre-defined criteria of safety and efficacy . Manufacturing human grade AAV vectors for phase I/II*	UMMS

*** Not started**