Active now	Year Awarded	Investigator(s)	Institution	Project	Type of Project
**	2018	Tony Futerman, PhD	Weizmann Institute of Science, Israel	Role of microglia in Sandhoff disease pathology	basic research
**		Alessandra d'Azzo, PhD	St. Jude Children's Research Hospital	Role of the Plasma membrane-ER Contact Sites in GM1-mediated Neuronal Cell Death	basic research
**		Xuntian Jiang, PhD	Washington University	Oligosaccharide Biomarkers for Disease Progression and AAV Therapeutic Efficacy in GM1 Gangliosidosis	biomarkers
**	2017	Miguel Sena-Esteves, PhD	U Massachusetts Medical Center	Accelerated program for CSF delivery of AAV gene therapy for Tay-Sachs and Sandhoff patients (off cycle)	gene therapy
		Miguel Sena-Esteves, PhD	U Massachusetts Medical Center	Amendment to 2015 grant for Pre-Clinical studies (off-cycle)	
**	2017	Alessandra Biffi, MD	Children's Hospital - Boston	Proof of concept study of HSC gene therapy for Tay-Sachs disease gene and cell therapy	gene and cell therapy
**		Heather Gray-Edwards, PhD	Auburn	Minimally invasive delivery of AAV gene therapy in the Tay-Sachs Sheep gene therapy	gene therapy
**		Tim Wood, PhD and Stephane Demotz, PhD	Greenwood Genetics & Dorphan	Development of a quantitative method for the determination of a pentasaccharide in GM1-gangliosidosis patient cells to assess the potential therapeutic efficacy of a beta-galactosidase pharmacological chaperone drug candidate	biomarkers
**	2016	Bev Davidson, PhD	Children's Hospital of Philadelphia	Identifying Novel Therapeutics for Treating GM2 Gangliosidoses	small molecules
**		Angela Gritti, PhD	San Raffaele Scientific Institute, Italy	Novel combined gene/cell therapy strategies to provice full rescue of the Sandhoff pathological phenotype	gene and cell therapy
**		Martin Grootveld,	De Montfort University (Leicester, UK)	Rapid Identification of New Biomarkers for the Classification of GM1 and GM2 Gangliosidoses: A HNMR-linked Metabolomics Strategy	biomarker
**		Cynthia Tifft, MD, PhD	NIH	Clinically Relevant Outcome Measures for Patients with Late Onset Tay-Sachs disease Ascertained Real-Time Through Patient	clinical trial readiness
		Doug Martin, PhD	Auburn	Lipid Biomarkers of Tay-Sachs Disease	biomarkers
	2015	Miguel Sena-Esteves, PhD	U Massachusetts Medical Center	Pre-clinical studies of AAVrh8-Hex gene delivery in TSD	gene therapy (TSGT Consortium)
**	2015	Denis Lehotay, PhD	University of Saskatchewan	Development and Validation of an MS-MS Method for the Detection of Hexosaminidase Deficiency in Tay-Sachs	clinical trial readiness
		Doug Martin, PhD	Auburn University College of Veterinary Medicine	Intravascular gene therapy for feline GM2 gangliosidosis	gene therapy (TSGT Consortium)
**		Heather Lau, MD, MS Paola Leone, PhD	New York University	Defining the Natural History of Canavan Disease through Development of an International Registry	clinical trial readiness
**		Florian Eichler, MD	Massachusetts General Hospital	Clinical Trial Readiness for Late Onset Tay-Sachs	clinical trial readiness
		Eric Sjoberg, PhD	OrPhi Therapeutics	Generation of a knock-in mutant *Hexb* mouse model	animal model (late onset GM2)
	2014	David Radin, PhD	BioStrategies, LC	Lectin-assisted transnasal delivery of corrective enzyme for GM1 gangliosidosis	new therapeutic approach
	2013	Miguel Sena-Esteves, PhD	U Massachusetts Medical Center	Selection of a new AAVrhB vector design and safety testing in NHP	gene therapy (TSGT Consortium)
		Alessandra d'Azzo, PhD	St. Jude Children's Research Hospital	Studies of the molecular and biochemical bases of neurodegeneration in sialidosis	basic research
		Doug Martin, PhD	Auburn University	Breeding Flock for the Sheep Model of Tay-Sachs Disease",	gene therapy (TSGT Consortium)
		Doug Martin, PhD	Auburn University	Cat pathology studies (70% CTSF / 30% NTSAD)	gene therapy (TSGT Consortium)
	2012		UC Davis	Vector Manufacturing (70% CTSF / 30% NTSAD)	gene therapy (TSGT Consortium)
		Doug Martin, PhD	Auburn University	Supplemental Pre-Clinical Studies of AAV Gene Therapy in Feline Sandhoff Disease	gene therapy (TSGT Consortium)
		Doug Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 3 (70% CTSF / 30% NTSAD)	gene therapy (TSGT Consortium)
			U of Florida	Supplemental equipment for tox studies	gene therapy (TSGT Consortium)
	2011	Fran Platt, PhD / Allie Colaco	University of Oxford	Validation of a Potential Biomarker for the GM1 and GM2 Gangliosidoses	biomaker
		Doug Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 2	gene therapy (TSGT Consortium)
		Florian Eichler, MD	Massachusetts General Hospital	Clinical Outcome Measures for a Gene Therapy Trial in Infantile and Juvenile GM2	natural history study (TSGT Consortium)
		Guangping Gao, PhD	University of Massachusetts Medical	Optimization of Efficacious Gene Therapy for Canavan Disease	gene therapy
		Yu-Tah Li, PhD	Tulane University	Studies of Taurine-Conjugated GM2 in Tay-Sachs Disease	novel marker
	2010	Fran Platt, PhD	University of Oxford	Optimizing the Therapeutic Potential of Anti-inflammatory Therapy in Tay-Sachs and Related Diseases: Targeting lL-1β Generated by	small molecules
		Maria Traka, PhD	University of Chicago	Development of an in vitro approach to identify molecular pathways of Canavan disease	basic research
		Jean-Pyo Lee, PhD / Evan Y. Snyder, MD,	Tulane University	The Therapeutic Potential of Human Induced Pluripotent Stem Cells (IPSCs) in the Sandhoff Disease Mouse Model of Lysosomal Storage	stem cell therapy
		Gustavo Maegawa, PhD	Johns Hopkins University	Developing a High Throughput Screening Assay to Identify Potential Drugs for Metachromatic Leukodystrophy	small molecules

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		Doug Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 1	gene therapy (TSGT Consortium)
	2009	Alexey Pshezhetsky, PhD	Universite of Montreal	Novel therapy for Tay-Sachs disease, sialidosis and galactosialidosis using a metabolic bypass catalyzed by the lysosomal sialidase Neu4	novel marker
		Mark Sands, PhD	Washington University	Combination Therapy for Krabbe Disease	combination therapy
		Joe Clarke, MD, PhD	Hospital for Sick Children	Proposed Investigator-Initiated Clinical Trial of Pyrimethamine as a Treatment for Late-Onset GM2 gangliosidosis (Tay-Sachs and	phase I clinical trial
		Florian Eichler, MD	Massachusetts General Hospital	A Biomarker for Disease Progression in GM2 and other Neurolipidoses	biomarker
		Edwin Kolodny, MD	NYU	Proposed Investigator-Initiated Clinical Trial of Pyrimethamine as a Treatment for Late-Onset GM2 gangliosidosis (Tay-Sachs and	phase I clinical trial
	2008	Stephanos Kyrkanides, PhD	Stony Brook University	Retrograde transfer of therapeutic vectors enabled by the trigeminal sensory system	gene therapy
		Angela Gritti, PhD / Alessandra Biffi, PhD	San Raffaele	Evaluation of Combined Approaches Using Hematopoietic and Neural Stem Cells for the Treatment of Globoid Cell Leukodystrophy	combination therapy (stem cells)
	2007	Florian Eichler, MD	Massachusetts General Hospital	The Natural History of Tay-Sachs Disease	gene therapy (TSGT Consortium)
		Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated Gene Therapy for Tay-Sachs Disease: Vector Selection for Preclinical Development	gene therapy (TSGT Consortium)
		Timothy Cox, MD M. Begoña Cachón-	University of Cambridge	Pre-Clinical/Clinical Research Program: Tay-Sachs and Related Diseases	gene therapy (TSGT Consortium)
		Douglas Martin, PhD	Auburn University	Pre-Clinical Studies of AAV Gene Therapy in Feline GM2 Gangliosidosis	gene therapy (TSGT Consortium)
		Thomas Seyfried, PhD	Boston College	Neurochemical and Immunological Evaluation of AAV Gene Therapy Strategies	gene therapy (TSGT Consortium)
	2007	Susan L. Cotman, PhD	Massachusetts General Hospital	Small molecule screening to identify modifiers of lysosomal trafficking, a putative therapy for Batten disease	small molecules
		Doug Martin, PhD	Auburn University	Pre-clinical gene therapy for GM2 in a feline model	gene therapy (TSGT Consortium)
		Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated gene therapy for Tay-Sachs: Vector selection for pre-clinical development	gene therapy (TSGT Consortium)
		Aryan Namoodiri, PhD	Uniformed Services University of the Heath	Preclinical Research toward Acetate Supplementation Therapy for Canavan Disease	small molecules
	2004	James A. Shayman, MD	University of Michigan	High throughput screening for inhibitors of ganglioside GM2 synthase	small molecules
	2003	Jean-Pyo Lee, PhD/Evan Y. Snyder,	Beth Israel Deaconess Medical Center/Burnham	Therapeutic Potential of Neural Stem Cells in the Gangliosidoses (Tay-Sachs & Sandhoff Diseases)	stem cell therapy
		Cynthia Tifft, MD, PhD	Children's Research Institute of Children's	Comprehensive Biochemical Analysis of Cerebrospinal Fluid in Patients with GM2 Storage Disorders:Molecular Pathogenesis of	biomarkers
	2002	Bruce A. Bunnell, PhD	Tulane University	In utero Gene Therapy of Sandhoff Disease in a Murine Model	gene therapy
		Stephanos Kyrkanides., PhD	University of Rochester School of Medicine &	Perinatal Gene Therapy for β-hexosaminidase disorders (Tay-Sachs and Sandhoff diseases)	gene therapy
		Paola Leone, PhD	University of Medicine and Dentistry of New Jersey	Neuroprotective Effect of Minocycline in Sandhoff Disease	small molecules
		Thomas N. Seyfried, PhD	Boston College	Therapeutic evaluation of NB-DGJ for ganglioside storage diseases	substrate reduction
				TOTAL	$ 4,015,998
				LSD Research Consortium	$ 100,000
				Total including NTSAD's LSDRC	$ 4,115,998
				TSGT	$ 2,059,728
					50%