	Year Awarded	Investigator(s)	Institution	Project	Type of Project
1	2013	Alessandra d'Azzo, PhD	St. Jude Children's Research Hospital	**Studies of the molecular and biochemical bases of neurodegeneration in sialidosis**	basic research
2		Doug Martin, PhD	Auburn University	**Cat pathology studies (70% CTSF / 30% NTSAD)**	gene therapy (TSGT Consortium)
3	2012		UC Davis	**Vector Manufacturing (70% CTSF / 30% NTSAD)**	gene therapy (TSGT Consortium)
4		Doug Martin, PhD	Auburn University	**Supplemental Pre-Clinical Studies of AAV Gene Therapy in Feline Sandhoff Disease**	gene therapy (TSGT Consortium)
5		Doug Martin, PhD	Auburn University	**Sheep as a Model of Tay-Sachs Disease - Year 3 (70% CTSF / 30% NTSAD)**	gene therapy (TSGT Consortium)
6			U of Florida	Supplemental equipment for tox studies	gene therapy (TSGT Consortium)
7	2011	Fran Platt, PhD / Allie Colaco	University of Oxford	Validation of a Potential Biomarker for the GM1 and GM2 Gangliosidoses	biomaker
8		Doug Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 2	gene therapy (TSGT Consortium)
9		Florian Eichler, MD	Massachusetts General Hospital	**Clinical Outcome Measures for a Gene Therapy Trial in Infantile and Juvenile GM2**	natural history study (TSGT Consortium)
10		Guangping Gao, PhD	University of Massachusetts Medical School	Optimization of Efficacious Gene Therapy for Canavan Disease	gene therapy
11		Yu-Tah Li, PhD	Tulane University	Studies of Taurine-Conjugated GM2 in Tay-Sachs Disease	novel marker
12	2010	Fran Platt, PhD	University of Oxford	Optimizing the Therapeutic Potential of Anti-inflammatory Therapy in Tay-Sachs and Related Diseases: Targeting IL-1β Generated by Aberrant NLRP3 Inflammasome	small molecules
13		Maria Traka, PhD	University of Chicago	Development of an in vitro approach to identify molecular pathways of Canavan disease	basic research
14		Jean-Pyo Lee, PhD / Evan Y. Snyder, MD, PhD	Tulane University	The Therapeutic Potential of Human Induced Pluripotent Stem Cells (IPSCs) in the Sandhoff Disease Mouse Model of Lysosomal Storage Disorders.	stem cell therapy
15		Gustavo Maegawa, PhD	Johns Hopkins University	**Developing a High Throughput Screening Assay to Identify Potential Drugs for Metachromatic Leukodystrophy**	small molecules
16		Doug Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 1	gene therapy (TSGT Consortium)
17	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
18		Mark Sands, PhD	Washington University	Combination Therapy for Krabbe Disease	combination therapy
19		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 Sandhoff Disease)	phase I clinical trial
20		Florian Eichler, MD	Massachusetts General Hospital	A Biomarker for Disease Progression in GM2 and other Neurolipidoses	biomarker
21		Edwin Kolodny, MD	NYU	Proposed Investigator-Initiated Clinical Trial of Pyrimethamine as a Treatment for Late-Onset GM2 gangliosidosis (Tay-Sachs and Sandhoff Disease)	phase I clinical trial
22	2008	Stephanos Kyrkanides, PhD	Stony Brook University	Retrograde transfer of therapeutic vectors enabled by the trigeminal sensory system	gene therapy
23		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)
24	2007	Florian Eichler, MD	Massachusetts General Hospital	The Natural History of Tay-Sachs Disease	gene therapy (TSGT Consortium)
25		Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated Gene Therapy for Tay-Sachs Disease: Vector Selection for Preclinical Development	gene therapy (TSGT Consortium)
26		Timothy Cox, MD M. Begoña Cachón-	University of Cambridge	Pre-Clinical/Clinical Research Program: Tay-Sachs and Related Diseases	gene therapy (TSGT Consortium)
27		Douglas Martin, PhD	Auburn University	Pre-Clinical Studies of AAV Gene Therapy in Feline GM2 Gangliosidosis	gene therapy (TSGT Consortium)
28		Thomas Seyfried, PhD	Boston College	Neurochemical and Immunological Evaluation of AAV Gene Therapy Strategies	gene therapy (TSGT Consortium)
29	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
30		Doug Martin, PhD	Auburn University	Pre-clinical gene therapy for GM2 in a feline model	gene therapy (TSGT Consortium)
31		Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated gene therapy for Tay-Sachs: Vector selection for pre-clinical development	gene therapy (TSGT Consortium)
32		Aryan Namoodiri, PhD	Uniformed Services University of the Heath Sciences	Preclinical Research toward Acetate Supplementation Therapy for Canavan Disease	small molecules
33	2004	James A. Shayman, MD	University of Michigan	High throughput screening for inhibitors of ganglioside GM2 synthase	small molecules
34	2003	Jean-Pyo Lee, PhD/Evan Y. Snyder, MD, PhD	Beth Israel Deaconess Medical Center/Burnham Institute	Therapeutic Potential of Neural Stem Cells in the Gangliosidoses (Tay-Sachs & Sandhoff Diseases)	stem cell therapy
35		Cynthia Tifft, MD, PhD	Children's Research Institute of Children's National Medical Center	Comprehensive Biochemical Analysis of Cerebrospinal Fluid in Patients with GM2 Storage Disorders:Molecular Pathogenesis of Disease Progression	biomarkers
36	2002	Bruce A. Bunnell, PhD	Tulane University	In utero Gene Therapy of Sandhoff Disease in a Murine Model	gene therapy
37		Stephanos Kyrkanides., PhD	University of Rochester School of Medicine & Dentistry	Perinatal Gene Therapy for β-hexosaminidase disorders (Tay-Sachs and Sandhoff diseases)	gene therapy
38		Paola Leone, PhD	University of Medicine and Dentistry of New Jersey	Neuroprotective Effect of Minocycline in Sandhoff Disease	small molecules
39		Thomas N. Seyfried, PhD	Boston College	Therapeutic evaluation of NB-DGJ for ganglioside storage diseases	substrate reduction