National Tay-Sachs & Allied Diseases Association

Past Grant Recipients (2002-2022)

Year Awarded	Investigator(s)	Institution	Project	Type of Project	Off-Cycle Grant
2022	Michael Gelb, PhD Hamid Khaledi, PhD	GelbChem	Newborn Screening Assay Development (bridge grant)	diagnostics	P
2022	Elise Townsend, DPT, PhD, PCS	Massachusetts General Hospital	Construction and Validation of the Infantile GM2 Rating Scale (off-cycle)	clinical trial readiness	P
2022	Amanda Gross, PhD	Auburn University	Dual site administration of AAV gene therapy for treatment of feline GM1 gangliosidosis	gene therapy	
2018	Tony Futerman, PhD	Weizmann Institute of Science, Israel	Role of microglia in Sandhoff disease pathology	basic research	
2018	Alessandra d'Azzo, PhD	St. Jude Children's Research Hospital	Role of the Plasma membrae-ER Contact Sites in GM1-mediated Neuronal Cell Death	basic research	
2018	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	P
2017	Miguel Sena-Esteves, PhD	U Massachusetts Medical Center	Amendment to 2015 grant for Pre-Clinical studies (off-cycle)		P
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	
2017	Heather Gray-Edwards, PhD	Auburn University	Minimally invasive delivery of AAV gene therapy in the Tay-Sachs Sheep gene therapy	gene therapy	
2017	Tim Wood, PhD Stephane Demotz, PhD	Greenwood Genetic Center, South Carolina & Dorphan, Switzerland	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	Beverly Davidson, PhD	Children's Hospital, Philadelphia	Identifying Novel Therapeutics for Treating GM2 Gangliosidoses	gene and cell therapy	
2016	Angela Gritti, PhD	San Raffaele Scientific Institute, Italy	Novel combined gene-cell therapy strategies to provide full rescue of the Sandhoff pathological phenotype		
2016	Martin Grootveld	De Montfor University, Leicester, UK	Rapid Identification of New Biomarkers for the Classification of GM1 and GM2 Gangliosidoses: A coupled ^{1}H NMR-and LC/MS-Linked Metabolomics Strategy	biomarkers	
2016	Cynthia Tifft, MD, PhD	National Institutes of Health (NIH)	Clinically Relevant Outcome Measures for Patients with Late Onset Tay - Sachs disease Ascertained Real-Time Through Patient Wearable Technology	clinical trial readiness	
2016	Douglas Martin, PhD	Auburn University	Lipid Biomarkers of Tay-Sachs Disease	biomarkers	
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	
2015	Douglas Martin, PhD	Auburn University	Intravascular gene therapy for feline GM2 gangliosidosis	gene therapy (TSGT Consortium)	
2015	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	
2015	Florian Eichler, MD	Massachusetts General Hospital	Clinical Trial Readiness for Late Onset Tay-Sachs	clinical trial readiness	
2015	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)	
2013	Alessandra d'Azzo, PhD	St. Jude Children's Research Hospital	Studies of the molecular and biochemical bases of neurodegeneration in sialidosis	basic research	
2013	Douglas Martin, PhD	Auburn University	Breeding Flock for the Sheep Model of Tay-Sachs Disease	gene therapy (TSGT Consortium)	
2013	Douglas 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)	
2012	Douglas Martin, PhD	Auburn University	Supplemental Pre-Clinical Studies of AAV Gene Therapy in Feline Sandhoff Disease	gene therapy (TSGT Consortium)	
2012	Douglas Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 3 (70% CTSF / 30% NTSAD)	gene therapy (TSGT Consortium)	
2012	University 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	biomarkers	
2011	Douglas Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 2 (70% CTSF / 30% NTSAD)	gene therapy (TSGT Consortium)	
2011	Florian Eichler, MD	Massachusetts General Hospital	Clinical Outcome Measures for a Gene Therapy Trial in Infantile and Juvenile GM2	natural history study (TSGT Consortium)	
2011	Guangping Gao, PhD	U Massachusetts Medical Center	Optimization of Efficacious Gene Therapy for Canavan Disease	gene therapy	
2011	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 IL-1β Generated by Aberrant NLRP3 Inflammasome Activation	small molecules	
2010	Maria Traka, PhD	University of Chicago	Development of an in vitro approach to identify molecular pathways of Canavan disease	basic research	
2010	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	
2010	Gustavo Maegawa, PhD	Johns Hopkins University	Developing a High Throughput Screening Assay to Identify Potential Drugs for Metachromatic Leukodystrophy	small molecules	
2010	Douglas Martin, PhD	Auburn University	Sheep as a Model of Tay-Sachs Disease - Year 1	gene therapy (TSGT Consortium)	
2009	Alexey Pshezhetsky, PhD	University of Montreal	Novel therapy for Tay-Sachs disease, sialidosis and galactosialidosis using a metabolic bypass catalyzed by the lysosomal sialidase Neu4	novel marker	
2009	Mark Sands, PhD	Washington University	Combination Therapy for Krabbe Disease	combination therapy	
2009	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 1 clinical trial	
2009	Florian Eichler, MD	Massachusetts General Hospital	A Biomarker for Disease Progression in GM2 and other Neurolipidoses	biomarkers	
2009	Edwin Kolodny, MD	New York University	Proposed Investigator-Initiated Clinical Trial of Pyrimethamine as a Treatment for Late-Onset GM2 gangliosidosis (Tay-Sachs and Sandhoff Disease)	phase 1 clinical trial	
2008	Stephanos Kyrkanides, PhD	Stony Brook University	Retrograde transfer of therapeutic vectors enabled by the trigeminal sensory system	gene therapy	
2008	Angela Gritti, PhD Alessandra Biffi, PhD	San Raffaele Scientific Institute, Italy	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)	
2007	Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated Gene Therapy for Tay-Sachs Disease: Vector Selection for Preclinical Development	gene therapy (TSGT Consortium)	
2007	Timothy Cox, MD M. Begoña Cachón-Gonzalez	University of Cambridge	Pre-Clinical/Clinical Research Program: Tay-Sachs and Related Diseases	gene therapy (TSGT Consortium)	
2007	Douglas Martin, PhD	Auburn University	Pre-Clinical Studies of AAV Gene Therapy in Feline GM2 Gangliosidosis	gene therapy (TSGT Consortium)	
2007	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	
2007	Douglas Martin, PhD	Auburn University	Pre-clinical gene therapy for GM2 in a feline model	gene therapy (TSGT Consortium)	
2007	Miguel Sena-Esteves, PhD	Massachusetts General Hospital	AAV-mediated gene therapy for Tay-Sachs: Vector selection for pre-clinical development	gene therapy (TSGT Consortium)	
2007	Aryan Namoodiri, PhD	Uniformed Services University	Preclinical Research toward Acetate Supplementation Therapy for Canavan Disease	small molecules	
2004	James A. Shayman	University of Michigan	High throughput screening for inhibitors of ganglioside GM2 synthase	small molecules	
2003	Jean-Pyo Lee, PhD Evan Y. Snyder, MD	Beth Isreal Deaconess Medical Center / Burnham	Therapeutic Potential of Neural Stem Cells in the Gangliosidoses (Tay-Sachs & Sandhoff Diseases)	stem cell therapy	
2003	Cynthia Tifft, MD, PhD	Children's Hospital National Medical Center	Comprehensive Biochemical Analysis of Cerebrospinal Fluid in Patients with GM2 Storage Disorders: Molecular Pathogenesis of Disease Progression	biomarkers	
2002	Bruce A. Bunnell, PhD	Tulane University	In utero Gene Therapy of Sandhoff Disease in a Murine Model	gene therapy	
2002	Stephanos Kyrkanides, PhD	University of Rochester School of Medicine & Dentistry	Perinatal Gene Therapy for β-hexosaminidase disorders (Tay-Sachs and Sandhoff diseases)	gene therapy	
2002	Paola Leone	University of Medicine and Dentistry of New Jersey	Neuroprotective Effect of Minocycline in Sandhoff Disease	small molecules	
2002	Thomas Seyfried, PhD	Boston College	Therapeutic evaluation of NB-DGJ for ganglioside storage diseases	substrate reduction	