

2017 FARA Awarded Grants

Principal Investigator/Organization	Grant Description	Type of Research	Co-funding
Drug Discovery			
Robert B. Wilson - Children's Hospital of Philadelphia	Center of Excellence: Drug Discovery	Basic/Translational	Cure FA Foundation & Hamilton & Finneran Family
David Corey - University of Texas Southwestern	Development of Oligonucleotide Activators of FXN Expression	Translational	
Richard Wade-Martins - University of Oxford	Identification of FXN-increasing drug targets by CRISPR-mediated mutagenesis	Basic/Translational	
Gino Cortopassi - University of California Davis; Paola Giunti - University College London; Mark Pook - Brunel University	<i>2016-2017 Bronya J. Keats International Research Collaboration Award:</i> Drug rescue of frataxin-dependent neural and cardiac pathophysiology in FA models.	Basic/Translational	
Hugo Bellen - Baylor College of Medicine	Suppressing the iron/sphingolipid/PDK/Mef2 pathway implicated in FA for therapeutic evaluation	Basic/Translational	Cure FA Foundation
Gene & Stem Cell Therapy			
Benjamin Deverman - California Institute of Technology	Evaluating Novel Capsid Engineered for Efficient CNS Transduction, as Frataxin Gene Deliver Vehicles	Translational	FARA Australia
Lead Candidates			
	<i>2016-2017 Kyle Bryant Translational Research Award :</i>		
RaNA/Q-state	A human iPSC-based cardiac model of Friedreich's Ataxia for drug discovery and patient stratification using all optical electrophysiology.	Translational/Clinical	
Alastair Wilkins - University of Bristol	Stem Cell Mobilizing Therapy: Cytokine G-CSF, Pilot Clinical Trial	Translational/Clinical	Ataxia UK
	<i>2015-2016 Kyle Byrant Translational Research Award :</i>		
Catabasis	Evaluation of CAT-4001 in Frataxin-deficient mouse models and DRG neurons to enable its therapeutic development to treat Friedreich's Ataxia	Translational	
Sanjay Bidichandani - University of Oklahoma	Is FXN DNA Methylation a determinant of response to HDAC inhibitor treatment in Friedreich ataxia?	Basic/Translational	
Mechanisms of Pathways of Disease			
	<i>2016-2017 Bronya J. Keats International Research Collaboration Award:</i>		
Dolores Molto - University of Valencia, Spain	Identification of genetic factors involved on FXN transcriptional silencing mediated by the GAA repeat expansion	Basic	FARA Ireland
Javier Santos - University of Buenos Aires	Structural dynamics and the consolidation of protein function in protein complexes involved in the biosynthesis of iron-sulfur clusters: Quaternary addition of small Trojan tutor proteins	Basic	
Katia Aquilano - University of Rome Tor Vergata	Studying the role of brown fat in Friedreich's ataxia	Basic	
Zhen Yan - University of Virginia School of Medicine	Endurance and resistance exercise mitigate Friedreich's ataxia	Translational	
Arnulf Koeppen - Veteran Affairs Medical Center, Albany, NY	The pathogenesis of Friedreich ataxia	Basic/Translational	
Cell & Animal Models			
Joel Gottesfeld - The Scripps Research Institute	Development of a novel iPSC-derived neuronal cell model for Friedreich's ataxia: generation of stable doxycycline-inducible expression of progerin in FRDA neuronal cells (Fellowship: Baohu Ji, PhD)	Translational	FARA Ireland
Pierre Gilles Henry & Christophe Lenglet - University of Minnesota	Longitudinal MR Imaging and Spectroscopy at 9.4T in a Conditional Mouse Model of FA	Translational	Cure FA Foundation
Cat Lutz - The Jackson Laboratories	Standardization and Characterization of Mouse models for the Study of FA	Basic/Translational	
Natural Hx & Biorespository			
David Lynch - Children's Hospital of Philadelphia; Susan Perlman - University of California Los Angeles; George Wilmot - Emory University; Kathy Mathews - University of Iowa; Sub Subramony - University of Florida; Chad Hoyle - Ohio State University; Grace Yoon - Sick Kids; Martin Delatycki - Murdoch Children's Research Institute	Collaborative Clinical Research Network in FA - Clinical Site Activity for Natural History and Biorepository	Clinical	
Theresa Zesiewicz - University of South Florida	Collaborative Clinical Research Network in FA - University of South Florida site activity, Symposium, Biomarker and Clinical Research	Clinical	
Christian Rummey, Clinical Data Science GmbH	Biostatistician working on Collaborative Clinical Research Network in FA data set	Clinical	
David Lynch - Children's Hospital of Philadelphia	Center of Excellence - Translational & Clinical Research	Translational/Clinical	Cure FA Foundation & Hamilton & Finneran Family
Outcome Measures & Biomarkers			

Pierre Gilles Henry & Christophe Lenglet - University of Minnesota	Early and Longitudinal Assessment of Neurodegeneration in the Brain and Spinal Cord in Friedreich's Ataxia	Clinical	
Matthew Hirschey - Duke University	Protein biomarkers in FRDA cardiomyopathy to monitor disease progression and therapeutic efficacy	Translational	
Manuela Corti - University of Florida	Clinical outcome measures of efficacy in the treatment of Friedreich's ataxia	Clinical	
Ian Blair - University of Pennsylvania	Center of Excellence - Metabolic Biomarkers	Translational	Cure FA Foundation & Hamilton & Finneran Family
Pierre Gilles Henry - University of Minnesota	Measurement of TCA Cycle Rate in the Dentate Nucleus in Friedreich's ataxia	Clinical	Cure FA Foundation
David Lynch - Children's Hospital of Philadelphia; Massimo Pandolfo - Erasme University Hospital	Biomarker Consortium: Neurophysiologic Biomarkers in FA	Clinical	Voyager Therapeutics
David Herrmann - University of Rochester	Biomarker Consortium: In vivo confocal imaging of Meissner's Corpuscles as a biomarker in FA	Clinical	Voyager Therapeutics
Martin Delatycki - Murdoch Children's Research Institute, Melbourne, Australia; Theresa Zesiewicz - University of South Florida	Biomarker Consortium: Longitudinal gait and balance measurement	Clinical	Voyager Therapeutics & Agilis
Cardiac Research			
Martin Delatycki - Murdoch Children's Research Institute, Melbourne Australia; Kim Lin - Children's Hospital of Philadelphia	Interstitial fibrosis, the renin-angiotensin-aldosterone system and biomarkers in the cardiac disease of Friedreich ataxia	Clinical	FARA Australia
Kim Lin - Children's Hospital of Philadelphia	Center of Excellence - cardiac research and educational	Clinical	Cure FA Foundation & Hamilton & Finneran Family