

2016 FARA Awarded Grants

Principal Investigator/Organization	Grant Description	Type of Research	Co-funding
Drug Discovery			
Andrew Dancis - University of Pennsylvania	A therapeutic strategy for Friedreich's ataxia: Frataxin bypass by enhancing the mitochondrial cysteine desulfurase activity	Translational	
Pfizer	High throughput screen of Pfizer small molecule chemical library in FRDA patient-derived cells to look for regulators of frataxin protein	Basic/Translational	
Robert Wilson - University of Pennsylvania	(1) Center of Excellence - Drug discovery (2) Drug testing and development for FA	Basic/Translational	Hamilton & Finneran Family
Omar Khmour - Arizona State University	Approaches to correct mitochondrial abnormalities and synaptic pathology in frataxin-deficient sensory neurons	Basic/Translational	
David Corey - University of Texas Southwestern	Development of Oligonucleotide Activators of FXN Expression	Basic/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 Award:</i> Drug rescue of frataxin-dependent neural and cardiac pathophysiology in FA models	Basic/Translational	FARA Ireland
Gene & Stem Cell Therapy			
Helene Puccio - INSERM, IGBMC, France	The development of gene therapy approach for the treatment of FRDA	Translational	The National Ataxia Foundation
Sathiji Nageshwaran - Harvard University	Fellowship award: Epigenetic editing of the frataxin gene: a novel therapeutic approach for FA.	Basic	FARA Ireland
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	
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 Bronya J. Keats International Research Award:</i>			
Dolores Molto - University of Valencia, Spain	Identification of genetic factors involved on FXN transcriptional silencing mediated by the GAA repeat expansion	Basic	
Jordi Magrané - Weill Cornell Medical College	Analysis of mitochondrial dynamics in cultured neurons and in in vivo mouse models of Friedreich's Ataxia	Basic/Translational	
Arnulf Koeppen - Veteran Affairs Medical Center, Albany, NY	FA Tissue Bank and Autopsy Program	Basic/Translational	
Cell & Animal Models			
Mirella Dottori - University of Melbourne, Australia	Transplantation studies of sensory neurons derived from Friedreich Ataxia induced pluripotent stem cells into the dorsal root ganglia	Basic/Translational	FARA Ireland
Cat Lutz - The Jackson Laboratories	Standardization and Characterization of Mouse models for the Study of FA	Basic/Translational	
Natural Hx & Biorepository			
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	
David Lynch - Children's Hospital of Philadelphia	Center of Excellence - Translational & Clinical Research	Translational/Clinical	Hamilton & Finneran Family
Theresa Zesiewicz - University of Southy 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	
Outcome Measures & Biomarkers			
Matthew Hirschey - Duke University	Protein biomarkers in FRDA cardiomyopathy to monitor disease progression and therapeutic efficacy	Translational/Clinical	
Henry Pierre Gilles & Christophe Lenglet - University of Minnesota	Early and Longitudinal Assessment of Neurodegeneration in the Brain and Spinal Cord in Friedreich's Ataxia	Clinical	Ataxia UK & GoFAR

Marek Napierala & Jill Butler - University of Alabama, Birmingham	Defining the molecular signature of FA to identify novel biomarkers	Translational	FARA Ireland
Elisabetta Sorangi - The Scripps Research Institute	Identification of FRDA gene expression biomarkers for Phase II/III efficacy trials in FA	Translational	
Manuela Corti - University of Florida	Clinical outcome measures of efficacy in the treatment of Friedreich's ataxia	Clinical	FARA Ireland
Ian Blair - University of Pennsylvania	Center of Excellence - Metabolic Biomarkers	Translational	Hamilton & Finneran Family
David Lynch - Children's Hospital of Philadelphia; Massimo Pandolfo - Erasmus 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
Cindy Casaceli - University of Rochester	Biomarker Consortium: Database	Clinical	
Cardiac Research			
Helene Puccio - INSERM, IGBMC, France	<i>2016-2017 Keith Michael Andrus Cardiac Research Award:</i> Investigation of cardiac pathophysiological mechanism and relevant biomarker in the context of FXN deficiency and FXN overexpression induced toxicity	Translational	
Alice Pebay - University of Melbourne, Australia	<i>2015-2016 Keith Michael Andrus Cardiac Research Award:</i> Screening of phenotypic abnormalities in Friedreich's ataxia-induced pluripotent stem cell-derived CMs	Translational	
Kim Lin - Children's Hospital of Philadelphia	Center of Excellence - cardiac research and educational	Clinical	Hamilton & Finneran Family
Erin Seiffert - Thomas Jefferson University	<i>2016 Keith Michael Andrus Cardiac Research Award:</i> Impact of Frataxin deficiency on cardiac substrate metabolism	Translational	
Martin Delatycki & Kim Lin - Murdoch Children's Research Institute, Melbourne Australia & Children's Hospital of Philadelphia	Interstitial fibrosis, the renin-angiotensin-aldosterone system and biomarkers in the cardiac disease of Friedreich ataxia	Clinical	FARA Australia
Mark Payne & Matthew Hirschey - Indiana University & Duke University	Mitochondrial Protein Acetylation and Heart Failure in FA	Basic/Translational	FARA Ireland
Other			
The National Ataxia Foundation	Ataxia Investigator Meeting	Scientific Conference	