

2015 FARA Awarded Grants

Principal Investigator/Organization	Grant Description	Type of Research	Co-funding
Drug Discovery			
Veronique Monnier - Université Paris Diderot	<i>2014 Keith Michael Andrus Cardiac Research Award:</i> Identification of therapeutic compounds on a cardiac Drosophila model of Friedreich's ataxia	Translational	FARA Ireland
Andrew Dancis - University of Pennsylvania	A therapeutic strategy for Friedreich's ataxia: Frataxin bypass by enhancing the mitochondrial cysteine desulfurase activity	Translational	
Edward L. Grabczyk - Louisiana State University	Small molecule induced exon skipping of MLH3 to slow repeat expansion in an FRDA mouse model	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	
Gene & Stem Cell Therapy			
Helene Puccio - INSERM, IGBMC, France	Development of neuronal gene therapy for the treatment of FRDA	Translational	FARA Ireland
Ricardo Pinto Mouro - Harvard University	CRISPR/Cas9-based Therapeutics for Friedreich's ataxia	Basic/Translational	FARA Ireland & The National Ataxia Foundation
Joel Gottesfeld - The Scripps Research Institute	Stem Cell Therapeutics for Friedreich's ataxia (Postdoctoral Fellowship: Baohu Ji)	Translational	
Lead Candidates			
Miriam Cnop - Université Libre de Bruxelles	Incretin Analogs as new therapeutics for FA	Clinical	
Retrotope	Patient travel costs for RT001 clinical trial	Clinical	
Paola Giunti - University College London	Investigation of mitochondrial function and novel therapies in Friedreich's ataxia mouse models	Translational	GoFAR
Catabasis	<i>2015-2016 Kyle Byrant Translational Research Award:</i> Evaluation of CAT-4001 in Frataxin-deficient mouse models and DRG neurons to enable its therapeutic development to treat Friedreich's Ataxia	Clinical	
Mechanisms of Pathways of Disease			
Alain Martelli - CERBM-GIE/IGBMC, France	Primary dorsal root ganglia sensory neurons with complete or partial loss of frataxin as models to investigate the neuropathophysiology of Friedreich ataxia	Basic	FARA Ireland
Massimo Pandolfo - Université Libre de Bruxelles	Investigation of the role of mTOR in Friedreich's ataxia and identification of new possible pathways for therapeutic intervention.	Basic	FARA Ireland
Zhen Yan - University of Virginia School of Medicine	Exercise impacts on mitochondria and muscle function in Friedreich's ataxia	Basic/Translational	
Hugo Bellen - Baylor College of Medicine	The role of iron accumulation and increased lipid synthesis in the pathogenesis of Friedreich's ataxia	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	
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 Australia
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; Christopher Gomez - University of Chicago; 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	
David Lynch - Children's Hospital of Philadelphia	Center of Excellence - Translational & Clinical Research	Translational/Clinical	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	Ataxia UK & GoFAR
Ian Blair - University of Pennsylvania	Center of Excellence - Metabolic Biomarkers	Translational	Hamilton & Finneran Family

Elizabetta Sorangi - The Scripps Research Institute	Gene Expression Studies in FA	Translational	
Marek Napierala & Jill Butler - University of Alabama, Birmingham	Defining the molecular signature of FA to identify novel biomarkers	Translational/Clinical	FARA Ireland
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
Cindy Casaceli - University of Rochester	Biomarker Consortium: Database	Clinical	
Cardiac Research			
<i>2015 Keith Michael Andrus Cardiac Research Award:</i>			
Alice Pebay - University of Melbourne, Australia	Screening of phenotypic abnormalities in Friedreich's ataxia-induced pluripotent stem cell-derived CMs	Translational	FARA Ireland
Kim Lin - Children's Hospital of Philadelphia	Center of Excellence - cardiac research and education	Clinical	Hamilton & Finneran Family
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
Other			
All India Institute of Medical Sciences	FA Scientific Conference	Scientific Conference	