

2014 FARA Awarded Grants

Principal Investigator/Organization	Grant Description	Type of Research	Co-funding
Drug Discovery			
Robert B. Wilson - University of Pennsylvania	Center of Excellence: Drug Discovery	Basic/Translational	Hamilton & Finneran Family
<i>2014 Keith Michael Andrus Cardiac Research Award :</i>			
Veronique Monnier - Université Paris Diderot	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	
David Corey - University of Texas Southwestern	Activation of Frataxin Expression by Duplex RNA	Translational	
Gene & Stem Cell Therapy			
Helene Puccio - INSERM, IGBMC, France	The development of gene therapy approach for the treatment of FRDA	Translational	FARA Ireland
Javier Diaz-Nido - Universidad Autónoma de Madrid	Development of novel blood-brain barrier crossing DNA nanocarriers to treat FA	Translational	FARA Ireland & GENE FA
Joel Gottesfeld - The Scripps Research Institute	Stem Cell Therapeutics for Friedreich's ataxia	Translational	
Lead Candidates			
Paola Giunti - University College London	Investigation of mitochondrial function and novel therapies in Friedreich's ataxia mouse models	Translational	GoFAR, FARA Ireland
David Lynch - Children's Hospital of Philadelphia; Cindy Casceli - University of Rochester	Open-label, pilot study of interferon gamma (Actimmune™) for the treatment of Friedreich's ataxia	Clinical	
Mechanisms of Pathways of Disease			
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	
Michele Lufino - University of Oxford	Visual Dissection of GAA-mediated mechanisms of FRDA repression and identification of novel candidate factors involved in frataxin function	Basic	FARA Ireland & Ataxia UK
Joel Gottesfeld - The Scripps Research Institute	Role of Sirtuins in FA (PostdoctoralFellowship: Li/Ji)	Basic	
Stephen Dzul & Tim Stemmler - Wayne State University	Characterizing Isu Scaffolding Protein and ISC Multiprotein Complex Structure and Function In Vitro	Basic	American Heart Association
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	
Cell & Animal Models			
Vijayendran Chandran - University of California Los Angeles	Generating cellular and mouse model for Friedreich's ataxia via gene expression	Basic/Translational	
Mirella Dottori - University of Melbourne, Australia	Modelling Friedreich Ataxia Neurodegeneration using Induced Pluripotent Stem Cells	Basic/Translational	FARA Australia
Cat Lutz - The Jackson Laboratories	Standardization and Characterization of Mouse models for the Study of FA	Basic/Translational	GoFAR
Natural Hx & Biorepository			
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 (CCRN) in FA - Clinical Site Activity for Natural History and Biorepository	Clinical	
Theresa Zesiewicz - University of South Florida	Collaborative Clinical Research Network (CCRN) 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			

Mark Baker - Newcastle University	Beta-band EMG-EMG coherence: a novel, painless and simple screening test for the onset of corticospinal tract disease/dorsal root ganglionopathy in Friedreich's ataxia	Clinical	Ataxia UK
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
Ian Blair - University of Pennsylvania	Center of Excellence - Metabolic Biomarkers	Clinical	Hamilton & Finneran Family
Elizabetta Sorangi - The Scripps Research Institute	Gene Expression Studies in FA	Clinical	
Max Little - Aston University, Birmingham, UK	Smartphone app - pilot study with Children's Hospital of Philadelphia & Collaborative Clinical Research Network in FA	Clinical	
Cardiac Research			
Kim Lin - Children's Hospital of Philadelphia	Center of Excellence - cardiac research and educational	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
Mark Payne - Indiana University; Matthew Hirschey - Duke University	<i>2013-2014 Keith Michael Andrus Cardiac Research Award: Mitochondrial Protein Acetylation and Heart Failure in FA</i>	Basic/Translational	FARA Ireland
Other			
National Ataxia Foundation	Ataxia Investigator Meeting	Scientific Conference	
Federation of American Societies for Experimental Biology (FASEB)	Mitochondrial Biogenesis and Dynamics in Health, Disease, and Aging	Scientific Conference	