

**2014 FARA Awarded Grants**

Principal Investigator/Organization	Grant Description	Type of Research	Co-funding
<b>Drug Discovery</b>			
Robert B. Wilson - University of Pennsylvania	Center of Excellence: Drug Discovery	Basic/Translational	Hamilton & Finneran Family
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	
David Corey - University of Texas Southwestern	Activation of Frataxin Expression by Duplex RNA	Translational	
<b>Gene &amp; Stem Cell Therapy</b>			
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	
<b>Lead Candidates</b>			
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	
<b>Mechanisms of Pathways of Disease</b>			
Jordi Magrané - Weill Cornell Medical College	Analysis of mitochondrial dynamics in cultured neurons and 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	
<b>Cell &amp; Animal Models</b>			
Vijayendran Chandran - University of California Los Angeles	Generating cellular and mouse model for Frideich'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
<b>Natural Hx &amp; Biorespository</b>			
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
<b>Outcome Measures &amp; Biomarkers</b>			

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	
<b>Cardiac Research</b>			
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
<b>Other</b>			
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	