

## 2013 FARA Funded Grants

Principal Investigator/Organization	Grant Description	Type of Research	Focus Area/Outcome
	<b>Drug Discovery</b>		
Mark Pook - Brunel University	Investigation of diazoxide as a novel frataxin-increasing therapy for Friedreich ataxia	Translational	Drug Discovery
Roberto Testi - University of Rome	Investigating New Therapeutic Approaches in Friedreich's ataxia - ubiquitin-competing molecules	Translational	Drug Discovery
Marek Napierala - University of Alabama	Novel compounds alleviating transcriptional silencing in Friedreich's Ataxia - NIH HTS	Translational	High Throughput Screening/Drug Discovery
Miriam Cnop - Université Libre de Bruxelles, 2013-2014 Kyle Bryant Translational Research Award	Incretin analogs as new therapeutics for Friedreich's ataxia	Translational	Drug Discovery
Sidney Hecht - Arizona State University	Advanced Generation of Multifunctional Radical Quenchers	Translational	Drug Discovery
Mark Pook - Brunel University	HMTase inhibitors as a novel epigenetic-based therapeutic approach for FRDA	Translational	Drug Discovery
Robert Wilson - University of Pennsylvania	A Nutrient Therapeutic Approach to Friedreich's Ataxia	Translational	Drug Discovery
	<b>Gene and Stem Cell Therapy</b>		
Mark Pook and Michael Themis - Brunel University	An investigation to determine the efficacy and safety of lentivirus FXN gene delivery	Translational	Gene and Stem Cell Therapy
Joel Gottesfeld and Carlos Barbas, The Scripps Research Institute	Stem Cell Therapeutics for Friedreich's ataxia	Translational	Gene and Stem Cell Therapy
Marek Napierala, University of Alabama	Correction of the Friedreich's ataxia gene defect using zinc finger nucleases	Translational	Gene and Stem Cell Therapy
Jonathan Jones, University Miguel Hernandez, 2012-2013 New Investigator Award	Neuroprotective effect of bone marrow-derived stem cells in a Friedreich's ataxia mouse model	Basic/Translational	Gene and Stem Cell Therapy
Hélène Puccio, Centre Européen de Recherche en Biologie et en Médecine	The development of gene therapy approach for the treatment of FRDA	Translational	Gene and Stem Cell Therapy
Javier Diaz-Nido, Fundacion Severo Ochoa & Ernest Giralt, Institut De Recerca Biomedica	Development of novel blood-brain barrier crossing DNA nanocarriers to treatFA	Basic/Translational	Gene and Stem Cell Therapy
	<b>Lead Candidates / Drug Development</b>		
James Rusche, RepliGen	HDACi RGFP963 and Metabolite characterization and analysis	Translational	Drug Development
Robert Molinari, Retrotope	Safety Study in Rat of isotopically modified PUFAs to decrease oxidative stress in Friedreich ataxia	Translational	Drug Development
David Lynch, Children's Hospital of Philadelphia & Cindy Casaceli, University of Rochester	Open-label, pilot study of interferon gamma (Actimmune™) for the treatment of Friedreich's ataxia	Translational	Drug Development
	<b>Mechanism or Pathway of Disease</b>		
Michele Lufino, Oxford University	Visual Dissection of GAA-mediated mechanisms of FRDA repression and identification of novel candidate factors involved in frataxin function	Basic	Mechanisms or Pathway of Disease
Joel Gottesfeld, The Scripps Research Institute	Role of mismatch repair enzymes in FA repeat expansion - Fellowship grant	Basic	Mechanisms or Pathway of Disease
Isabelle Iltis, Christophe Lenglet & Pierre-Gilles Henry, University of Minnesota, 2012-2013 Kyle Bryant Translational Research Award	Mapping the anatomical and functional connectivity of the Central Nervous System in Friedreich's Ataxia using Magnetic Resonance Imaging	Clinical	Mechanisms or Pathway of Disease
Jordi Magrene, Cornell University	Analysis of mitochondrial dynamics in cultured neurons and in vivo mouse models of Friedreich's Ataxia	Basic	Mechanisms or Pathway of Disease
Edward Grabczyk, Louisiana State University	The role of somatic repeat expansion in FRDA disease progression	Basic	Mechanisms or Pathway of Disease

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	<b>Cell &amp; Animal Models</b>		
Massimo Pandolfo, Université Libre de Bruxelles	Characerization of Friedreich's ataxia iPSC-derived neurons	Translational	Cell & Animal Models
Vijay Chandran, University of California Los Angeles, 2011-2012 New Investigator Award	Generating cellular and mouse model for Friedreich's ataxia via gene expression	Translational	Cell & Animal Models
Mirella Dottori, University of Melbourne	Modelling Friedreich Ataxia Neurodegeneration using Induced Pluripotent Stem Cells	Translational	Cell & Animal Models
Cat Lutz, The Jackson Laboratories	Standardization and Characterization of Mouse models for the Study of FA	Translational	Cell & Animal Models
	<b>Collaborative Clinical Research Network in Friedreich's Ataxia (CCRN in FA)</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 in Friedreich's Ataxia (CCRN in FA) - Clinical Site Activity for FA-Clinical Outcome Measures, Natural History and Biorepository	Clinical	Natural History, Outcome Measures and Trial Planning
Cindy Casceli, University of Rochester	CCRN in FA - Data Management, Analysis & Clincial Coordination	Clinical	Natural History, Outcome Measures and Trial Planning
Theresa Zesiewicz, University of South Florida	USF CCRN in FA site activity, Symposium, Biomarker and Clinical Research	Clinical	Natural History and Clinical Research
	<b>Cardiac Research</b>		
Alice Pebay, Center for Eye Research Australia	Cardiac Differentiation of Friedreich ataxia-induced pluripotent stem cells for disease modelling	Basic	Cardiac
Mark Ziolo, The Ohio State University	Heart Failure in Friedreich's ataxia	Basic	Cardiac
Mark Payne, Indiana University & Matthew Hirshey, Duke University, 2013-2014 Keith Michael Andrus Cardiac Award	Mitochondrial Protein Acetylation and Heart Failure in FA	Basic	Cardiac
Arnulf Koeppen, Albany Medical Center	The pathogenesis of Friedreich cardiomyopathy	Basic	Cardiac
Martin Delatycki, Murdoch Children's Research Institute & Kim Lin, Children's Hospital of Philadelphia, 2013-2014 Bronya J Keats International Research Award	Interstitial fibrosis, the renin-angiotensin-aldosterone system and biomarkers in the cardiac disease of Friedreich ataxia	Translational	Cardiac
	<b>Other</b>		
	FASEB: Mitochondrial Biogenesis and Dynamics	Conference grant	
	7th International Conference on Fe-S Cluster Biogenesis and Regulation	Conference grant	