2012 FARA Funded Research Grants

Principal Investigator/Organization	Grant Description	Type of Research	Focus Area/Outcome
	Drug Discovery		
Mark Payne (in collaboration with Dr. Sidney Hecht) - Indiana University	Pilot Study: Testing Hecht Lead Compound in KO Mouse Model	Translational	Drug Discovery
Roberto Testi - University of Rome	Investigating New Therapeutic Approaches in Friedreich's ataxia - ubiquitin-competing molecules	Translational	Drug Discovery
Gino Cortopassi - University of California Davis, *2011-2012 Phillip Bennett Translational Research Award*	Repurposing existing approved drugs for Friedreich's ataxia therapy	Translational	High Throughput Screening/Drug Discovery
Marek Napierala - University of Texas, MD Anderson Cancer Center	Novel compounds alleviating transcriptional silencing in Friedreich's Ataxia - NIH HTS	Translational	High Throughput Screening/Drug Discovery
Sidney Hecht - Arizona State University	Advanced Generation of Multifunctional Radical Quenchers	Translational	Drug Discovery
	Gene and Stem Cell Therapy		
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
Joseph Sarsero, Murdoch Children's Research Institute, *2011-2012 Kyle Bryant Translational Research Award*	Correction of Friedreich ataxia iPS cells by non-viral gene therapy	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 Texas, MD Anderson Cancer Center	Correction of the Friedreich’s ataxia gene defect using zinc finger nucleases	Translational	Gene and Stem Cell Therapy
Jonathan Jones, University Miguel Hernandez, *2012 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
	Lead Candidates / Drug Development		
James Rusche, RepliGen	HDACi RGFP963 and Metabolite characterization and analysis	Translational	Drug Development
Juha Punnonen and Jeffrey Spencer, STATegics	Mechanism of Frataxin Enhancement by Recombinant Erythropoietin and Small Molecule Erythropoietin Mimetics	Translational	Drug Development

2012 FARA Funded Research Grants

Principal Investigator/Organization	Grant Description	Type of Research	Focus Area/Outcome
Robert Wilson (in collaboration with Retrotope) - University of Pennsylvania	Isotropic reinforecement of PUFAs to decrease oxidative stress in Freidreich's ataxia	Translational	Drug Development
	Mechanism or Pathway of Disease		
Michele Lufino, Oxford University	Visual Disection 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, 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 in vivo mouse models of Friedreich's Ataxia	Basic	Mechanisms or Pathway of Disease
	Cell & Animal Models		
Massimo Pandolfo,	Characerization of Friendreich'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 Fridreich's ataxia via gene expression	Translational	Cell & Animal Models
Cat Lutz, The Jackson Laboratories	Standardization and Characterization of Mouse models for the Study of FA	Translational	Cell & Animal Models
	Collaborative Clinical Research Network in Friedreich's Ataxia (CCRN in FA)		
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, 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

2012 FARA Funded Research Grants

Principal Investigator/Organization	Grant Description	Type of Research	Focus Area/Outcome
Theresa Zesiewicz, University of South Florida	USF CCRN in FA site activity, Symposium, Biomarker and Clinical Research	Clinical	Natural History and Clinical Researh
	Biomarkers		
David Lynch and Ian Blair, University of Pennsylvania	Mass spectrometric analysis of metabolic features of Friedreich Ataxia	Clinical	Biomarker
	Cardiac Research		
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
	Other		
National Ataxia Foundation	Ataxia Investigators Meeting - Conference grant	All	Multiple
Ataxia UK	2012 Ataxia Research Conference - Conference grant	All	Multiple
Bronya Keats, Australian National University	Advancing effective therapies for FA	All	Multiple