

**2011 FARA Funded Research Grants**

<b>Principal Investigator</b>	<b>Project Title / Description</b>	<b>Type of Research</b>	<b>Focus Area/Outcome</b>	<b>Co-funding</b>
<b>Drug Discovery</b>				
Sidney Hecht	Multifunctional Radical Quenchers for the Treatment of Friedreich's ataxia.	Translational	Drug Discovery	
Roberto Testi	Investigating New Therapeutic Approaches in Friedreich's ataxia - ubiquitin-competing molecules	Translational	Drug Discovery	
Robert Wilson	Novel Compounds for the treatment of Friedreich's ataxia	Translational	Drug Discovery	
Joel Gottesfeld	Second Generation Histone Deacetylase Inhibitors as Therapeutics for Friedreich's Ataxia	Translational	Drug Discovery	
Pierre Rustin	Identifying additional sensitive targets in Friedreich's ataxia	Translational	Drug Discovery	Multiple European Groups
<b>2011 Phillip Bennett Translational Resesarch Award</b>				
Gino Cortopassi	Repurposing existing approved drugs for Friedreich's ataxia therapy	Translational	HTS/Drug Discovery	
Ed Grabczyk	Transcription-coupled GAA-TCC expansion in human cells	Translational	HTS/Drug Discovery	
Devin Olgesbee	High-Throughput Meso-Scale Discovery of Frataxin Enhancers - cont'd from 2010 award	Translational	HTS/Drug Discovery	
<b>Gene and Stem Cell Therapy</b>				
Mark Pook & Michael Themis	An investigation to determine the efficacy and safety of lentivirus FXN gene delivery	Translational	Gene and Stem Cell Therapy	Ataxia UK
Joel Gottesfeld & Carlos Barbas	Stem Cell Therapeutics for Friedreich's ataxia	Basic	Gene and Stem Cell Therapy	
<b>Lead Candidates</b>				
Martin Delatycki	An open label proof of principle study of resveratrol as a treatment for FA - cont'd from 2010 award	Clinical	Lead Candidates	
Juha Punnonen - Stategics	Efficacy of small molecule erythropoietin mimetic compounds in enhancing frataxin expression in vitro and in vivo	Translational	Lead Candidates	
<b>2011 Keith Michael Andrus Memorial Award</b>				
Francisco Sacca	A double-blind, randomized, placebo-controlled, clinical trial to test the efficacy of Epoetin alfa on physical performance of FA patients	Clinical	Lead Candidates/Cardiac	
<b>Mechanism or Pathway of Disease</b>				
Joel Gottesfeld	Role of heterochromatin in triplet repeat expansion in Friedreich's ataxia.	Basic	Mechanism or Pathway of Disease	
Marcia Haigis	Investigating the role of sirtuins in FA - cont'd from 2010 award	Basic	Mechanism or Pathway of Disease	
<b>2010 - 2011 FARA New Investigator Award</b>				
Lata Mahishi	MicroRNAs in FA	Basic	Mechanism or Pathway of Disease/Genetic Modification	
Michelle Lufino	Visual Dissection of GAA-mediated mechanisms of FRDA repression and identification of novel candidate factors involved in frataxin function	Translational	Mechanism or Pathway of Disease	Ataxia UK, FARA-A
<b>Cell &amp; Animal Models</b>				
Helene Puccio	Modeling Friedreich ataxia by the development of induced pluripotent stem cells carrying (GAA) <sub>n</sub> pathogenic expansions - con't from 2010 award	Translational	Cell & Animal Models	
Mirella Dottori & Alice Pebay	Generation of induced pluripotent stem cells from FA patients	Translational	Cell & Animal Models	
Mirella Dottori	Developing a therapeutically viable system for generating FA iPS cell lines	Translational	Cell & Animal Models	
<b>2011 - 2012 FARA New Investigator Award</b>				
Vijay Chandran	Generating cellular and mouse model for Friedreich's ataxia via gene expression	Translational	Cell & Animal Models	
Mark Pook	Development of an improved GAA repeat expansion mouse model of Friedreich's ataxia	Translational	Cell & Animal Models	Ataxia UK
Jackson Labs	Standardization and Characterization of Mouse models for the Study of FA	Translational	Cell & Animal Models	
<b>Collaborative Clinical Research Network in FA</b>				
Multiple	CCRN in FA - Clinical Site Activity for Natural History and Biorepository	Clinical	Natural Hx & Biorepository	
University of Rochester	CCRN in FA - Data Management & Coordination supplement	Clinical	Natural Hx & Trial Planning	
<b>Outcome Measures &amp; Biomarkers</b>				

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Gary Rance	Translational measures of auditory function in individuals with FA	Clinical	Outcome Measures, Biomarkers & Improving Clin Outcomes	
David Lynch	Optical Coherence Tomography in FA	Clinical	Outcome Measures & Biomarkers	
Bruce Murdoch	Alteration of motor speech function as an indicator of progressive decline in the neurogenic function in individuals with FA	Clinical	Outcome Measures & Biomarkers	
<b>Cardiac Research</b>				
Alice Pebay	Cardiac tissue engineering from induced pluripotent stem cells from FA patients	Translational	Cardiac & Cell Models	
<b>2010 Keith Michael Andrus Memorial Award</b>				
Arnulf Koeppen	The cardiomyopathy of FA	Basic	Cardiac & Natural History	
<b>Improving Clinical Outcomes</b>				
Martin Delatycki	Clinical Management Guidelines	Clinical	Improving Clinical Outcomes	
Genetic Disease Screening Program / Mayo Clinic	Newborn screening test validation	Clinical	Improving Clinical Outcomes	
<b>Other</b>				
Bronya Keats	Advancing effective therapies for FA	Multiple	Other	