

## FUNDED GRANTS JANUARY- DECEMBER 2010

Principal Investigator	Project	Research Area	Category/Outcome	Co-funding
David Lynch	Collaborative Clinical Research Network for Friedreich's Ataxia – Bridge grants to individual sites as needed	Clinical	Clinical Outcome Measures, Biomarkers and Trials	MDA
Bernard Ravina	Supplement: Clinical Research Network for Friedreich's Ataxia – Data Coordination Center	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Theresa Zesiewicz	USF Ataxia Center; CCRN FA site	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Tetsuo Ashizawa & Sub Subramony	Collaborative Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Susan Perlman	Supplement: Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Theresa Zesiewicz	Double-blind, Randomized, Placebo-controlled pilot study in the Treatment of Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
University of Rochester: Clinical Trial Coordination Center	Data Management & Coordination Supplement: Double-blind, Randomized, Placebo-controlled pilot study in the Treatment of Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Tracey Rouault and Richard Holms	Development and use of synthetic iron-sulfur clusters in therapy of Friedreich ataxia	Translational	Iron-Sulfur Cluster assembly; ↓ oxidative stress and ↑ mitochondrial function	
Dottori & Pebay	Generation of induced pluripotent stem cells from Friedreich ataxia patients	Translational	Cellular models	FARA-A
Arnulf Koeppen	Friedreich's ataxia: Iron dysmetabolism in the central and peripheral nervous systems	Basic	Pathology & Iron Metabolism	
Marcia Haigis	Investigating the role of sirtuins in Friedreich's Ataxia	Basic/Translational	Mitochondrial; ↓ oxidative stress and ↑ mitochondrial function	
Edward Grabczyk	Transcription-coupled GAA.TCC expansion in human cells	Basic/Translational	FRDA gene; ↑ frataxin	
Helene Puccio	Modeling Friedreich ataxia by the development of induced pluripotent stem cells carrying (GAA) <sub>n</sub> pathogenic expansions	Translational	Cellular models	
Bronya Keats	Advancing effective therapies for FA	Basic/Translational/ Clinical	Multiple	FARA-A
Theresa Zesiewicz	Natural history biomarkers in FA	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Sidney Hecht	Multifunctional Radical Quenchers for the Treatment of Friedreich's Ataxia	Basic/Translational	Mitochondrial; ↓ oxidative stress and ↑ mitochondrial function	
Mark Pook	Development of an improved GAA repeat expansion mutation-based mouse model of Friedreich ataxia for therapeutic testing.	Translational	Animal models	Go-FAR
Pierre Rustin	Identifying Additional Sensitive Targets in Friedreich's ataxia	Translational	Drug Discovery	Multiple European Groups
Gary Rance	Translational Measures of Auditory Function in Individuals with Friedreich Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
Mirella Dottori	Developing a Therapeutically Viable System for Generating Friedreich Ataxia induced-Pluripotent Stem Cell Lines	Translational	Cellular Models	

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Brigitte Sturm	Efficacy Of Small Molecule Erythropoietin Mimetic Compounds In Enhancing Frataxin Expression In Normal And Frataxin-Deficient Cells	Translational	Drug Discovery	
Joel Gottesfeld	Role of Heterochromatin in Triplet Repeat Expansion in Friedreich's Ataxia	Basic	FRDA gene	
Myriam Rai	Development of histone deacetylase inhibitors as treatment for Friedreich's ataxia	Translational	FRDA gene; ↑ frataxin	Go-FAR
National Ataxia Foundation	Ataxia Investigators Meeting, March 2010	All	Multiple	Multiple
RepliGen	Identification of Backup compound for HDAC inhibitor RG2833 to Treat Friedreich's Ataxia	Translational	Drug Development; ↑ frataxin	Go-FAR
Joel Gottesfeld	Second Generation Histone Deacetylase Inhibitors as Therapeutics for Friedreich's Ataxia	Translational	Drug Development; ↑ frataxin	
Edison	EPI-743 – Preclinical development and manufacturing	Translational	Drug Development; Mitochondrial; ↓ oxidative stress and ↑ mitochondrial function	
The Jackson Laboratories	Standardization and Characterization of Mouse Models for the Study of FA	Translational	Animal Models	
Michelle Lufino	Visual dissection of GAA-mediated mechanisms of <i>FRDA</i> repression and identification of novel candidate factors involved in frataxin function	Basic/Translational	FRDA gene; ↑ frataxin	Ataxia UK FARA - A
Robert Wilson	Novel Compounds for the treatment of Friedreich ataxia	Translational	Drug Development; Mitochondrial; ↓ oxidative stress and ↑ mitochondrial function	
Alice Pebay	Cardiac tissue engineering from induced pluripotent stem cells from Friedreich ataxia patients	Translational	Cellular models and engineering	
<b>2009 - 2010 FARA New Investigator Award</b>				
Marguerite Evans-Galea	Evaluating the Molecular and Epigenetic Alternations in Friedreich Ataxia	Basic	FRDA gene; ↑ frataxin	
<b>2010 - 2011 FARA New Investigator Award</b>				
Lata Mahishi	MicroRNAs in Friedreich ataxia	Basic/Translational	FRDA gene; ↑ frataxin	
<b>2009 - 10 American Heart Association/Friedreich's Ataxia Research Alliance Cardiology Research Award</b>				
Jonathan Joseph Silberg	Split protein biosensors for imaging mitochondrial iron-sulfur clusters	Basic/ Translational	Iron-Sulfur Cluster assembly; ↓ oxidative stress and ↑ mitochondrial function	AHA
<b>2010 Kyle Bryant Translational Research Award</b>				
Devin Oglesbee	High-Throughput Meso-Scale Discovery of Frataxin Enhancers	Translational	Drug Discovery	
Martin Delatycki	An open label proof of principle study of resveratrol as a treatment for Friedreich ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	
<b>2010 Keith Michael Andrus Cardiac Research Memorial Award</b>				
Arnulf Koeppen	The cardiomyopathy of Friedreich's ataxia	Basic	Pathology and Cellular Function	