

## FUNDED GRANTS JANUARY- DECEMBER 2009

Principal Investigator	Project	Research Area	Category	Outcome	Co-funding
David Lynch	Collaborative Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All	MDA
Bernard Ravina	Supplement: Clinical Research Network for Friedreich's Ataxia – Data Coordination Center	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All	
Martin Delatycki	Supplement: Collaborative Clinical Research Network for Friedreich's Ataxia - Australia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All	FARA-A
Theresa Zesiewicz	Double-blind, Randomized, Placebo-controlled pilot study in the Treatment of Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All	
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	All	
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	↑ frataxin	FARA-A
Thierry Morlet	Auditory & Vestibular Neuropathies in Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All	
Mark Pook	Development of an improved GAA repeat expansion mutation-based mouse model of Friedreich ataxia for therapeutic testing.	Translational	Animal models	All	Go-FAR
Arnulf Koeppen	Friedreich's ataxia: Iron dysmetabolism in the central and peripheral nervous systems	Basic	Pathology & Iron Metabolism	Elucidate Pathophysiology	
Subha V. Raman, MD	Supplement: Preclinical Markers of Cardiomyopathy in Friedreich's Ataxia	Translational	Cardiac, Biomarkers	All	
Marcia Haigis	Investigating the role of sirtuins in Friedreich's Ataxia	Basic/Translational	Mitochondrial	↓ oxidative stress and ↑ mitochondrial function	

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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	All	
Bronya Keats	Advancing effective therapies for FA	Basic/Translational/Clinical	Multiple	All	
<b>2008 - 2009 FARA New Investigator Award</b>					
Alain Martelli	Iron Metabolism in FA: from the fundamental understanding of iron dysregulation to the therapeutic potential of iron chelators	Basic/Translational	Iron Metabolism	↓ oxidative stress and ↑ mitochondrial function	
<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>2008 - 09 American Heart Association/Friedreich's Ataxia Research Alliance Cardiology Research Award</b>					
R. Mark Payne	Use of TAT-Frataxin to Reverse the Cardiomyopathy of Friedreich's Ataxia	Basic/ Translational	Frataxin replacement & Cardiac	↑ frataxin	AHA
<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>2008 - 2009 Mitochondrial Therapeutic Approaches Research Award</b>					
Michael Koob	Towards Gene Therapy of Mitochondrial Diseases	Basic	Mitochondrial	↑ frataxin	MDA
<b>2009 Kyle Bryant Translational Research Awards</b>					
Mark Payne	Optimizing delivery of frataxin using cell penetrant peptides	Translational	Frataxin replacement	↑ frataxin	NAF
Marek Napierala	Crosstalk between microRNAs and iron metabolism in pathogenesis of Friedreich's ataxia.	Basic/Translational	FRDA gene expression & biomarkers	↑ frataxin	NAF
Gino Cortopassi	Screening for mitofunctional Friedreich's Ataxia therapeutics	Basic/Translational	Assay Dev't & Drug Discovery	↓ oxidative stress and ↑ mitochondrial function	NAF