

## FARA funds 17 grants, exceeding 1 Million in research dollars, in 2007.

FARA is excited to share that we approved for funding over 1 Million in research dollars for 17 grants and in addition our partner organizations funded an additional \$500,000 for these meritorious projects. FARA's competitive research grant program accepts grants on a continuous basis throughout the year from FA scientists around the world. All grant applications go through a scientific peer-review process to ensure that we fund the best research. In 2007, FARA awarded 17 grants, which exceed \$1,500,000 in funding.

Over the past year FARA has prioritized funding to support research that directly advances drug targets (translational research) and clinical trials. This type of research requires higher levels of funding. Our ability to fund research is directly dependent on your active support through donations and participation in fundraising. FARA partners are also committed to advancing FA research and we have been able to partner with them in making several of these grant awards. We would like to recognize our 2007 funding partners:

Muscular Dystrophy Association  
National Ataxia Foundation and  
Friedreich's Ataxia Research Alliance – Australia/New Zealand.

Acting alone there is little we can accomplish but working together there is little we will **NOT** accomplish.

### Grants funded in 2007

Principal Investigator	Project	Research Area	Category	Outcome
*Joseph Sarsero	Development of pharmacological therapies for FA using humanized mouse models	Basic / Translational	Animal Model and Drug Screening	↑ frataxin
Pook/Gottesfeld	Histone Deacetylase Inhibitor (HDACI) therapy of a Friedreich's ataxia mouse model	Translational	Drug Development	↑ frataxin
*David Lynch	Collaborative Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
*Grazia Isaya	Modulators of frataxin assembly: development of high throughput screening agents	Translational assay	Drug Screening	All
Robert Wilson	Supplement to previous FARA grant and RO1 to support high throughput drug screening	Translational	Drug Screening	All
Marek Napierala	Influence of chlorambucil-conjugated GAA-TTC sequence-specific polyamides and Histone Deacetylase Inhibitors (HDACI) on repeat instability and frataxin expression	Basic / Translational	FRDA Gene	↑ frataxin
Filip Lim & Richard Wade-Martins	Neuron - validated approaches for developing Friedreich's ataxia therapeutics	Basic / Translational	FRDA Gene	↑ frataxin
Des Richardson	Iron Chelation Efficacy of Novel	Translational	Iron chelation	All

	PCIH Iron Chelators In Vivo and their Ability to Prevent the Pathology Observed in the Conditional Frataxin Knockout Mouse.		/ Drug Screening	
#Nuri Gueven	Use of a novel catalytic antioxidant, CTMIO, in a GAA repeat mouse model of FRDA	Translational	Antioxidants	↓ oxidative stress and ↑ mitochondrial function
Arnulf Koeppen	Friedreich's ataxia: Iron dysmetabolism in the central and peripheral nervous systems	Basic	Pathology & Iron Metabolism	Elucidate Pathophysiology
^Martin Delatycki	Supplement to Collaborative Clinical Research Network for Friedreich's Ataxia – Australia site	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
RepliGen Corporation	Preclinical optimization of HDAC inhibitors for treating Friedreich's Ataxia	Translational	FRDA gene	↑ frataxin
Robert Wilson	RNAi therapeutics for Friedreich ataxia	Basic/Translational	FRDA gene	↑ frataxin
*Erika Becker and Des Richardson	Mitochondrial Iron Overload and Friedreich's Ataxia: The Role of Frataxin in Iron Metabolism.	Basic	Frataxin function	Elucidate pathophysiology
Joel Gottesfeld	Improved HDAC inhibitors as Therapeutics for FA	Translational	FRDA gene	↑ frataxin
Bernard Ravina	Supplement Clinical Research Network for Friedreich's Ataxia – Data Coordination Center	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All

\* grants co-funded with MDA, # grant co-funded with NAF, ^ grant co-funded with FARA-Australia

"Research Area" refers to three broad categories of research -- basic, translational and clinical. Basic research is the most fundamental and explores the underlying causes and mechanisms (pathophysiology) of the disease. Translational (or preclinical) research advances the results of basic research from discovery through development, from "bench to bedside." It involves, for example, testing drug discoveries in animals and human cell cultures in preparation for tests in humans. Clinical research involves trials in humans. The "Category" column is used to characterize the focus of each research project. The "Outcome" column places the goal or long-term benefit of each project into one of four categories – Determine Pathophysiology, Improve Mitochondrial Function (↑), Reduce Oxidative Stress (↓), or Elevate Frataxin Protein levels (↑).