

FARA Grants Funded in 2005

Principal Investigator	Project	Research Area	Category	Outcome
Giovanni Coppola	Gene expression study of frataxin deficient mouse	Basic	Microarray analysis/gene expression	pathophysiology
Martin Delatycki	Ataxia Meeting, Nov 3 rd -4 th 2005, Australia	All	Collaborative Conference	All
Peter Dervan	Gene transcription therapy	Basic	FRDA gene	↑ frataxin
*Edison Pharmaceuticals	Development of drugs that increase mitochondrial function	Translational	Drug development	↑ mito function
Joel Gottesfeld	Polyamides (small molecules)	Basic	FRDA gene	↑ frataxin
Joel Gottesfeld	Chromatin structure and HDAC Inhibitors	Basic	FRDA gene	↑ frataxin
*Michael Hebert	Reporter system to measure transcription of sticky DNA	Basic/Translational	Assay development	↑ frataxin
* Panos Ioannou, Joseph Sarsero	Development of genomic reporter assays and humanized mouse models	Basic/Translational	Assay development	↑ frataxin
Grazia Isaya	Structure/Function studies of frataxin	Basic	Frataxin function	pathophysiology
Filip Lim and Richard Wade Martins	Gene therapy strategies using herpes virus amplicon vectors in transgenic mice	Translational	Gene therapy	↑ frataxin
Arnulf Koeppen	Iron & iron-responsive proteins in FA cardiomyopathy	Basic	Cardiology	pathophysiology
David Lynch, Tetsuo Ashizawa, Christopher Gomez, Susan Perlman, George Wilmot	Friedreich's ataxia clinical outcome measures – multi-center study	Clinical	Clinical outcome measures	All
David Lynch	Speech measures	Clinical	Clinical outcome measures	All
Bogdan Popescu, Wing Hang Tong	Bioiron meeting	All	Iron metabolism	All
Des Richardson	The role of iron and the use of chelation therapy	Basic	Iron metabolism	Pathophysiology ↓oxid stress
*Robert Wilson	Identification of drugs	Basic/Translational	Drug	All

	and drug targets	onal	screening	
John Day (NAF)	Ataxia Investigators Meeting, Tampa, FL	Clinical		All

*Funded by FARA and Seek A Miracle/MDA

Footnote: Grant awarded to Edison Pharmaceuticals was for \$3.4 million. In addition, FARA purchased \$1.1 million of Edison preferred stock. This grant/investment helped enable Edison, lacking the financial resources of a larger pharmaceutical company, to advance its promising compounds from drug discovery through drug development and prepare them for clinical trial. FARA will apply any investment dividends to additional FA research.

2-year grants funded in 2005 and 2006

Principal Investigator	Project	Research Area (basic, translational or clinical)	Category	Outcome (pathophysiology, ↑ mito function, ↑ frataxin, ↓oxid stress)
*Louise Cahill	Speech as indicator of decline in neurological function	Clinical	Clinical outcome measure	All
**Massimo Pandolfo	Overcoming transcription defect w/ small molecules	Translational	Animal and cellular models	↑ frataxin
*Annalisa Pastore	interaction between frataxin and Isc proteins	Basic	Frataxin function	pathophysiology
Jordi Tamarit	Protein damage induced by iron-overload in the yeast model of FA	Basic	Cell biology	pathophysiology
*Robert Wells	DNA triplexes in Friedreich's ataxia	Basic	FRDA gene	↑ frataxin

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