

Each Breakthrough

Brings us closer to a cure.

2009 Annual Report

Advancing Research for Friedreich's Ataxia

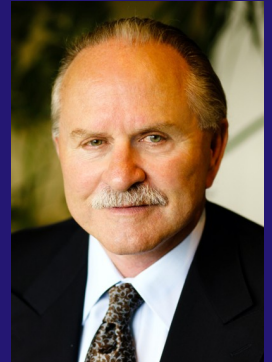
FARA

Friedreich's
Ataxia
Research
Alliance

From the Chairman

Dear Friends of FARA:

I am glad that I am not a scientist. In the two or so years that I have been involved with FARA, I have come to appreciate how difficult FA science is to do and to understand. Ron, Jen, and scientists such as Dave Lynch and Earl Giller have been patient with me, but it is clearly an uphill battle as is the battle against FA. One big difference is that the FA battle is being won.



Another year has gone by, and we are one year closer to the audacious goal FARA set for itself: To find a cure for FA by 2012. Much of the credit goes to the executive team – Ron, Jen, Juliann, and Felicia – and much more goes to our FARA families, personal and corporate friends of the families, and the cadre of scientists who have dedicated themselves to understanding and conquering Friedreich’s Ataxia.

Our two strategic initiatives – science and fundraising – have been in place for just over a year and are sowing dividends. We had our first clinical trial funded solely by FARA – a test of Varenicline – and we continue to broaden and deepen our research pipeline all over the world. More trials are underway and although never fast enough, we are making progress toward our goal and receiving high marks for the quality of the science characteristic of FA research.

Fundraising continues to improve. FARA was able to grow even in the worst economic environment in decades due to the efforts of patient families, friends, and corporate supporters. Outback Restaurant Partners, LLC set the standard for corporate generosity and was our Partner of the Year. It seems as though there is a local fundraising event somewhere almost every weekend thanks to the FARA families. And a special thanks to Paul and Suzanne Avery. It is through their leadership and generosity that the first Energy Ball came to life as a very successful fundraising event in Tampa Bay, Florida. The second Energy Ball is scheduled for this August. We hope that you will be able to help support this event.

Education and communication continue to be an important adjunct to success. As an “orphan disease” FA needs all the publicity it can get. On several fronts, that is exactly what we are doing. FARA is at the table with important organizations such as NIH, FDA, and other non-profits and drug companies. And we are in motion—literally, with Ride Ataxia and Race Across America—and Kyle Bryant. Kyle will tell you that his rides are fun rides, and they are for some. For others such as yours truly, they are a test of will and raise questions about Kyle’s grip on reality. But what cannot be questioned are their value to getting our name before the public and our disease a little better understood. Awareness and understanding are important ingredients to finding a cure.

Thanks to each of you for your support and inspiration,

Tom DeCotiis, Ph.D.

About FARA

The Friedreich's Ataxia Research Alliance (FARA) has been dedicated to advancing research for Friedreich's ataxia for over 11 years. In scientific research terms, that's not a lot of time. However, in that period, FARA has established a robust and admired program that has attracted the world's best scientists, advanced numerous treatment opportunities and forged collaborative relationships around the globe. FARA grants and activities provide support for basic and translational FA research, pharmaceutical/biotech drug development, clinical trials, and scientific conferences. FARA also serves as a catalyst between the public and scientific communities, to create worldwide exchanges of information that drive medical advances.

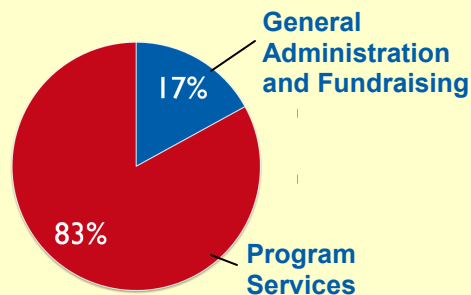
Mission

FARA's Mission is to marshal and focus the resources and relationships needed to cure FA by raising funds for research, promoting public awareness, and aligning scientists, patients, clinicians, government agencies, pharmaceutical companies and other organizations dedicated to curing FA and related diseases.

Fiscal Approach

A donor-supported organization, FARA allocates its funds in an ethical and responsible manner that exceeds the standards set for the non-profit industry. We are especially grateful to those who helped us advance our mission in fiscal year 2009 by contributing to revenues of \$3.5 million. As we expand our efforts, the board and staff remain vigilant about careful stewardship of FARA finances. In 2009, we have kept our fundraising and general operating and administrative expenses to about 17% of our overall revenue for the year. This is largely possible because of the significant volunteer and in-kind support we receive for our events.

How FARA funds were utilized in 2009



FARA Staff

Ron Bartek

Co-Founder and President

Jennifer Farmer, MS, CGC

Executive Director

Giovanni Manfredi, MD, PhD

Chief Scientific Officer

Bronya Keats, PhD

Chief Scientific Officer

Juliann Green

Chief Development Officer

Raychel Furr Bartek

Co-Founder and Patient- Family Liaison

Felicia DeRosa, MPA

Program Director

Kyle Bryant, Founder, Ride Ataxia

Ride Ataxia Director, FARA Spokesperson

Lyn Sgrillo

Executive Assistant

Join Our Mission

Learn how you can support FARA's efforts to treat and Cure FA at www.curefa.org/help.html

2009 Achievements

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Keeping our goals in focus



The Collaborative Clinical Research Network in FA (CCRN in FA) is a critical component of FARA's research program. Without it and the valuable patient information obtained through the clinic sites (10 as of 2009), our research would be nearly impossible. In 2009, The CCRN in FA:

- recruited and evaluated 100 new patients
 - saw more than 550 patients
 - provided data, advice and biological samples for planning and conducting numerous clinical trials and research studies
 - provided the infrastructure for two clinical trials
 - initiated scientific studies to evaluate new clinical outcome measures of speech, hearing and vision
 - grew the DNA bank to more than 300 samples enabling the first genetic modifier studies
 - hosted an FA Patient Symposium with over 150 patient families from the United States, Canada, Ireland, England and Austria in attendance.
-

FARA awarded 23 grants in 2009

Total 2009 research program = \$1,805,854

FA Patient Registry enrollment grew to 1,250

Insight from Jen Farmer, Executive Director

As FARA's Executive Director, I am responsible for ensuring that all of the programs, activities and operations of the organization support FARA's Strategic Initiatives and are carried out in the most efficient and effective manner. Our strategic initiatives are centered on advancing FA research, raising the funds necessary to accomplish our goals and communicating effectively with all constituents and the general public to ensure that FA is understood and considered in the rare disease dialogue.

While FARA's primary focus is on advancing FA research and bringing forward treatments, many other ancillary activities are critical to support and facilitate these goals including ongoing development of our research infrastructure and continuing to grow our revenues to match our increasing costs of research.

The Collaborative Clinical Research Network in FA is one major example of how vital it is for FARA to maintain its research infrastructure. Our scientific community, pharmaceutical partners and collaborators count on this infrastructure to support their research. As you'll read in the column to the left, the CCRN in FA made many significant contributions to the advancement of FA research in 2009. This was made possible through FARA funding, direction and leadership.

Here are just a few examples of how our research program expanded in 2009:

- We nearly doubled the number of grant applications reviewed in a single year
- Our research agenda was expanded to areas such as new forms of cell models and improved mouse models.
- FARA hosted an International FA therapeutics symposium which guided the FARA scientific advisory board in crafting our goals for 2010 and beyond. See next page for more detail.
- Most important, five of the lead candidates identified in FARA's strategic initiatives for advancement were in clinical trials this year. See the current FARA [Pipeline](#).
- Highlights of additional progress are included in this report. More information is available at www.curefa.org.

As we have from our beginnings, FARA relies heavily on the passion and commitment of our volunteers to help us carry out our mission. We could not accomplish all that we have in 2009 without the energy, expertise and hearts of many.

2009 Research Highlights

Kyle Bryant Translational Research Awards

In 2009, FARA and the National Ataxia Foundation (NAF) made three grant awards through the Kyle Bryant Translational Research Award Program:

Protein Replacement

Principal Investigator: Dr. Mark Payne, Indiana University School of Medicine—Optimizing delivery of frataxin using cell penetrant peptides

Drug discovery toward improving mitochondrial function

Principal Investigator: Dr. Gino Cortopassi, University of California, Davis—Screening for mitofunctional Friedreich's ataxia therapeutics

New strategies to regulate FRDA gene activity

Principal Investigator: Marek Napierala, University of Texas, MD Anderson Cancer Center—Crosstalk between microRNAs and iron metabolism in pathogenesis of Friedreich's ataxia

Therapeutics Symposium

More than 100 FA researchers and advocacy partners from around the world gathered in July 2009 for the FA Therapeutics Symposium in Philadelphia, PA. Presentations and discussions highlighted:

- progress in the development of previously identified therapeutic candidates, such as HDACI and TAT-Frataxin
- results from clinical trials including the Phase I study of A0001 and Phase III of Idebenone
- recent discoveries that point to new therapies
- advancements in new cell models, as well as drug discovery and development assays
- clinical research including biomarker studies and new clinical outcome measures.
- Formation of animal model task force

In addition to sharing progress and insights on various aspects of FA therapeutics and development, participants were encouraged to discuss and identify key assets that would accelerate progress significantly. Since the symposium, FARA has awarded research grants, formed task forces, and fostered collaborations and new partnerships to develop these assets.

Growing our Scientific Expertise

FARA believes in welcoming, mentoring and supporting new scientists in the FA research community. In 2009, FARA presented its New Investigator Award to Dr. Marguerite Evans- Galea of Murdoch Children's Research Institute, Australia.



Dr. Marguerite Evans-Galea

Dr. Evans-Galea has a strong background in molecular biology and genetics, which includes research on understanding oxidative stress response in biological systems and direct experience with developing gene therapy approaches for other genetic conditions. The title of the study she will conduct with the award is "Evaluating the Molecular and Epigenetic Alterations in Friedreich's Ataxia". Through this study, Dr. Evans-Galea will investigate the variability in FA and explore genetic-based explanations or controls, beyond the DNA sequence of the FRDA gene, for such variability.

FARA's unwavering commitment to collaboration has resulted in numerous scientific and funding partnerships around the world including:

AFAF—France
Ataxia UK—UK
AHA—US
CAFA—Canada
FEDAES—Spain
GoFAR—Italy

FARA—Australasia
FASI—Ireland
Euro Ataxia—Europe
MDA—US
NAF—US
NIH—US

Giving FARA and FA a Public Face



Growing Support for our Mission

In 2009, FARA's event-based fundraising program grew in size and number of events. FA families and their communities have continued to sustain critical core FARA funding through local events as they have done since FARA's beginning. Events organized and hosted by FA families in 2009 numbered well over 35 in 16 different states and included athletic and social gatherings.

Outback Steakhouse restaurants across the country also continued their generous fundraising activity in 2009. Their more than 25 events included luncheons, Heineken with a Heart happy hours, walks/runs, golf and sporting clays.

FARA added two of its own branded events in 2009—Ride Ataxia and the FARA Energy Ball. Ride Ataxia became FARA's family/community-focused fundraiser with a kick-off in the rolling hills of Philadelphia's suburbs. The one-day, multiple-distance format allowed people of all ages and abilities to participate. The first FARA Energy Ball gala in Tampa, FL welcomed 600 guests to three days of activities true to its namesake. Hosted by a dynamic and creative organizing committee in Tampa, the event kicked off with an educational symposium, included tennis and golf, and culminated in a gala dinner/auction.

FARA's Ambassador on wheels—Kyle Bryant

Friedreich's ataxia is a rare disease that struggles for attention because of small numbers. However, just like in our research, FARA is gaining momentum and recognition in the public eye. In October 2009, FARA offered me the opportunity to represent the cause as the FARA spokesperson and the director of our Ride Ataxia cycling events. Before officially joining the FARA team, I worked with FARA promoting events and raising funds. In the last quarter of 2009, I presented the FARA story to nine audiences including fundraisers, scientific conferences, business managers and schools. FARA has also experienced high visibility in online communities including our FaceBook Fan page introduced at the end of the year (As of mid-2010, we have more than 2200 fans) and YouTube videos that had over 9,000 views by the end of 2009 and the Ride Ataxia Blog which experienced 7,000 unique views from 95 different countries in 2008 and 2009. Public interest is building.

FARA's Ride Ataxia program began with a resounding success in Philadelphia on October 25, 2009. This ride included 350 participants, half of whom had never heard of Friedreich's ataxia. The participants also included eight members of the FA community on trikes, and representatives from 20 families affected by FA – from nearby and as far away as California.

2009 was a successful year in terms of raising awareness in the public eye and FARA looks forward to success in the future on the road toward the goal. Awareness is a community effort and I look forward to working with YOU to advance the FARA story. With your help, we will find a cure.

FARA fundraising event revenues exceeded \$2 Million in 2009, doubling the revenues from event-based fundraising of the previous year. This growth was due in large part to proceeds exceeding \$850,000 from the first annual FARA Energy Ball. Additionally, revenues from the two 2009 Ride Ataxia events exceeded \$390,000. We want to thank all of our fundraisers on behalf of the FA community. Your energy and dedication to FARA's mission provide the momentum propelling research forward.

Joint Venture Philanthropy—FARA style

A Model for Rare Disease Advocacy

From its early years, FARA has been recognized as a model for rare disease advocacy organizations. FARA supports our pharmaceutical partners with funding, patient recruitment, scientific expertise and advocacy with organizations such as the NIH and MDA.

A0001—Edison, Penwest, University of Pennsylvania

In 2005, FARA provided substantial support to a small biotech company, Edison Pharmaceuticals, that was advancing what it called the “next generation” of the CoQ10 enzyme. FARA led a collaborative effort along with Edison and Dr. Robert Wilson at the University of Pennsylvania to secure support from the NIH RAID (Rapid Access to Interventional Development) program to help fund the pre-clinical development of what would become A0001.

Subsequently, the compound was licensed to Penwest Pharmaceuticals who completed Phase I safety and dose-finding studies in 2009. FARA also organized and facilitated meetings between Penwest and FARA clinical investigators such as Dr. David Lynch to help plan for Phase II studies scheduled for 2010. (These studies are now in progress.)

FARA’s Patient Registry—adding value to our partnerships

In 2009, FARA’s patient registry supported recruitment of participants for clinical research by pharmaceutical partners and academic scientists. This registry is the critical link to successful research and is invaluable in enabling and accelerating FARA’s remarkably active clinical trial program. Most clinical trials fail—usually from insufficient patient participation. Drug companies often spend millions of dollars and a lot of time recruiting patients for trials. It is not unusual for companies to devote two years to recruitment. We cannot allow that to happen. FARA is urgently pursuing treatments and a cure and cannot spend years recruiting patients for trials. Nor can we permit trials to fail because of insufficient patient participation. Enter the FARA patient registry. With it, we have been able to recruit sufficient FA patients for clinical trials in weeks, not months or years, and none of the FA trials have failed for lack of sufficient participants.



Jubilant finishers from Ride Ataxia II — Portland to Seattle. The Ride resulted in \$360,000 for translational research.

Varenicline

2009 marked the first clinical trial funded by FARA. FARA acted quickly when Principal Investigator, Dr. Theresa Zesiewicz, at the University of South Florida CCRN in FA site found that symptoms of a cerebellar ataxia patient improved while taking Chantix®. Zesiewicz subsequently found similar results when treating patients with other types of ataxia, including Friedreich’s ataxia.

After a thorough review by FARA’s scientific committee, FARA proceeded to fund, for the first time on its own, a clinical trial to investigate varenicline (Chantix®), and did so at two FARA CCRN in FA sites, USF and the Children’s Hospital of Philadelphia. The trial continued into 2010. Regardless of final outcome, the trial identified a potential novel mechanism of action in the nervous system that may provide additional opportunities for treatment in FA.

Frequently Asked Questions

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FARA is focused on driving research that will lead to treatments and a cure for FA. Our program is held up as a model for other rare diseases due to our intense focus on an open, collaborative environment that speeds innovation. The pace of research is quickening. More researchers are joining the FARA team and more pharmaceutical companies are taking notice of our work. Below are answers to questions about FARA's scientific program and other common areas of interest of our supporters.

How does the FARA grant process work?

FARA has provided more than \$14 million in research funding over the past 12 years. Grants are provided for general research, to encourage new investigators focused on FA, for specific areas of FARA interest and for workshops and meetings.

All grant applications submitted to FARA are thoroughly reviewed in a professional and confidential "dual-review" process modeled after that of the NIH. First, FARA's Scientific Review Committee performs a preliminary review, followed by reviews by peers competent in the applicant's scientific area. Based on the peer-reviews, as well as the significance and relevance of the proposal to FARA's mission and strategic priorities, the Committee makes a recommendation to proceed to FARA's Board of Directors, which decides the level of funding to award to each meritorious grant. Within 60 days following completion of the grant period, Grantees are required to submit a report on the results. In some cases, interim reports are also required.

How does FARA decide what research to pursue?

In the early years, funding priorities were focused on understanding the causes of FA. While there is still more to learn about the intricacies of this disease, we have expanded our focus to translational and clinical research. You can review FARA's pipeline at www.curefa.org/pipeline.html

FARA's Grant Program Priorities

- Advance drug discovery and maintain a diverse treatment pipeline
- Facilitate drug development and translational research so that the most promising discoveries are rapidly brought to treatment trials
- Support the clinical research, infrastructure and biomarker discovery required to ensure effective and efficient clinical trials in a rare disease
- Support young/new researchers with innovative ideas and a commitment to FA research
- Reduce the morbidity and mortality caused by cardiac disease in FA

What is FARA doing to attract interest from the scientific and pharmaceutical community?

To maximize our presence in the research community, FARA attends and presents at professional conferences, addresses research groups, pharmaceutical companies, student and professional groups and other forums. We serve on numerous committees and advisory groups including those at the National Institutes of Health (NIH) and National Organization of Rare Diseases (NORD).

How does FARA use funds that are donated to the organization?

Donations support all of the programs described in this report, including scientific research, awareness and education, as well as the internal staffing and resources needed to operate the organization.

Why does FARA need to increase fundraising activity?

As FARA's research efforts have moved from the laboratory into the clinical setting, the costs of research have risen dramatically. In order to maintain our research momentum and lead our most promising treatments through drug development and clinical trials, we need more funding than FARA is currently receiving.

How can I stay more connected and informed on FARA's progress?

FARA provides timely information on our website, Facebook Fan page and through our e-mail notifications. Please join our mailing list to receive information and FARA news. There are options for research news, Ride Ataxia and for general FARA updates. You may unsubscribe at any time. If you are a patient, please make sure that you are subscribed to FARA's Patient Registry. You will receive notifications of clinical trials and scientific studies in which you may be qualified to participate.

FRIEDREICH'S ATAXIA RESEARCH ALLIANCE

STATEMENTS OF FINANCIAL POSITION

December 31, 2009 and 2008

	2009	2008
ASSETS		
CURRENT ASSETS		
Cash and cash equivalents	\$2,171,396	\$1,166,981
Receivables	85,374	44,621
Pledges receivable	50,000	-
Prepaid expenses	2,280	1,124
Investment in preferred stock	1,100,000	1,100,000
TOTAL CURRENT ASSETS	3,409,050	2,312,726
COMPUTER EQUIPMENT, net of accumulated depreciation of \$484 (2009) and \$21 (2008)	3,310	808
TOTAL ASSETS	\$3,412,360	\$2,313,534
LIABILITIES AND NET ASSETS		
CURRENT LIABILITIES		
Accounts payable	\$194,935	\$151
Accrued payroll liabilities	2,167	10,377
TOTAL CURRENT LIABILITIES	197,102	10,528
NET ASSETS		
Unrestricted	2,665,258	2,303,006
Temporarily restricted	550,000	-
TOTAL NET ASSETS	3,215,258	2,303,006
TOTAL LIABILITIES AND NET ASSETS	\$ 3,412,360	\$ 2,313,534

The independently audited financial statements of the Friedrich's Ataxia Research Alliance are available online at www.cureFA.org, or by contacting Friedrich's Ataxia Research Alliance, 102 Pickering Way, Suite 200, Exton, PA 19341/Phone: 484-875-3015

FARA Donor Bill of Rights

PHILANTHROPY is based on voluntary action for the common good. It is a tradition of giving and sharing that is primary to the quality of life. To ensure that philanthropy merits the respect and trust of the general public, and that donors and prospective donors can have full confidence in the Friedrich's Ataxia Research Alliance (FARA),

FARA declares that all donors have these rights:

- I. To be informed of FARA's mission, of the way FARA intends to use donated resources, and of its capacity to use donations effectively for their intended purposes.
- II. To be informed of the identity of those serving on FARA's governing board and staff leadership, and to expect the board and leadership to exercise prudent judgment in their stewardship responsibilities
- III. To have access to FARA's most recent financial statements.
- IV. To be assured their gifts will be used for the purposes for which they were given.
- V. To receive appropriate acknowledgement and recognition.
- VI. To be assured that information about their donations is handled with respect and with confidentiality to the extent provided by law.
- VII. To expect that all relationships with individuals representing FARA will be professional in nature.
- VIII. To be informed whether those seeking donations are volunteers or employees of FARA. FARA does not hire outside paid solicitors.
- IX. To have the opportunity for their names to be deleted from mailing lists
- X. To feel free to ask questions when making a donation and to receive prompt, truthful and forthright answers.

FRIEDREICH'S ATAXIA RESEARCH ALLIANCE

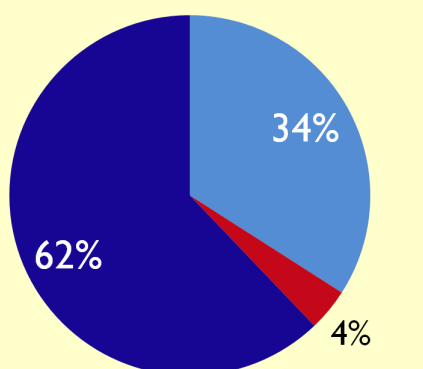
STATEMENTS OF ACTIVITIES

Years Ended December 31, 2009 and 2008

	2009	2008
UNRESTRICTED NET ASSETS		
Revenue and Support		
Contributions	\$654,018	\$945,525
Grants	134,672	64,628
Special Events	2,196,798	1,009,956
Interest Income	4,041	25,162
TOTAL REVENUE AND SUPPORT	2,989,529	2,045,271
Expenses		
Program Services		
Education, awareness and outreach	85,466	77,146
Patient registry	7,949	7,455
Research and grant program	1,805,854	1,282,775
Research conferences	115,827	70,346
TOTAL PROGRAM SERVICES	2,015,096	1,437,722
Supporting Services		
Fund-raising	248,466	59,250
Special events	305,186	109,158
General and administrative	58,529	55,997
TOTAL SUPPORTING SERVICES	612,181	224,405
TOTAL EXPENSES	2,627,277	1,662,127
CHANGE IN UNRESTRICTED NET ASSETS	362,252	383,144
TEMPORARILY RESTRICTED NET ASSETS		-
Contributions	550,000	
CHANGE IN NET ASSETS	912,252	383,144
NET ASSETS AT BEGINNING OF YEAR	2,303,006	1,919,862
NET ASSETS AT END OF YEAR	\$3,215,258	\$2,303,006

2009 Revenues (% of total revenues)

- Contributions
- Grants
- Special events



FARA's 2010 Leadership Team

2010 Board of Directors

Ronald J. Bartek, President/ Director/ Co-Founder

Retired U.S. Government Official; Business Consultant

Thomas A. DeCotiis, PhD, Chairman of the Board

Founder and Chief Executive Officer, CorVirtus

Marilyn E. Downing, Secretary

Teacher/Diagnostician, Special Education

Ed Ramsey, Treasurer

Business, Forest Products

Paul Avery

Chief Operating Officer, OSI Restaurant Partners, LLC, retired

Rob Dhoble

President, Diversified Agency Services, Omnicom

Earl Giller, MD, PhD, Scientific Director

Consultant, Global CNS Pharmacology Consulting, LLC

Holly Hedrick, MD

Surgeon, Children's Hospital of Philadelphia

Nicholas A. Johnson

Associate & Senior Mechanical Engineer,
Bard, Rao + Athanas Consulting Engineers, LLC, retired

Laura Kalick

Nonprofit Tax Director, BDO Seidman, LLP

Stephen Klasko, MD, MBA

Dean, University of South Florida College of Medicine
Senior Vice President, USF Health

Paul Marcotte

Attorney & Communications Consultant

Peter Pitts

Partner and Director, Global Healthcare, Porter Novelli
President and Co-Founder, Center for Medicine in the Public Interest

Scientific Advisory Board Co-Chairs

Giovanni Manfredi, MD, PhD

Chief Scientific Officer, FARA
Professor, Weill Cornell Medical College

Bronya Keats, PhD

Chief Scientific Officer, FARA
Professor, Australian National University

Board

Nicholas DiProspero, MD, PhD

Associate Director of Experimental Medicine
Johnson & Johnson

Grazia Isaya, MD, PhD

Professor of Biochemistry/Molecular Biology
and Pediatrics, Mayo Clinic Rochester

Arnulf Koeppen, MD

Professor of Neurology and Pathology
Albany Medical College

David Lynch, MD, PhD

Associate Professor of Neurology
Children's Hospital of Philadelphia/University
of Pennsylvania

Mark Payne, MD, FAAP, FACC

Professor of Pediatrics (Cardiology)
Indiana University School of Medicine

Bernard Ravina, MD, MS

Associate Professor of Neurology
University of Rochester

Giovanna Spinella, MD

Science and Program Consultant,
Former Director of External Research
NIH-Office of Rare Disease
Research

Robert Wilson, MD, PhD

Associate Professor of Pathology and Laboratory
Medicine, University of Pennsylvania

Advancing Research for Friedreich's Ataxia



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