



Fifth Annual **Friedreich's Ataxia Symposium** **Monday, October 8, 2012**

Crowne Plaza Valley Forge
King of Prussia, PA

Sponsored by the Friedreich's Ataxia Program at

 **The Children's Hospital of Philadelphia®**

Hope lives here.

Symposium Overview

Worldwide, Friedreich's Ataxia (FA) affects roughly one in 50,000 people, making it the most common in a group of related disorders called hereditary ataxias. While there is currently no cure for FA, efforts continue to be made towards research that will one day halt or even reverse disease progression. The Friedreich's Ataxia Program at The Children's Hospital of Philadelphia is pleased to present this one-day symposium providing patients and families with up-to-date clinical information, therapeutic approaches and current research being conducted in the field of Friedreich's Ataxia.

Meeting Location

The symposium will be held Monday, October 8, 2012 at the Crowne Plaza Valley Forge located in King of Prussia, Pa. Perfectly situated directly across from the King of Prussia Mall, the largest shopping mall in the United States. Valley Forge National Historic Park, the site of Washington's troops winter encampment during the Revolutionary war is also located less than two miles away.

Registration Information

Pre-registration is required and the registration deadline is September 24, 2012. Payment must accompany registration form for it to be processed. Once registration has been processed a confirmation letter will be mailed or faxed. PLEASE NOTE: Registration is not complete if you do not receive this confirmation. If you do not receive a confirmation within 10 days of registration, please contact us at (215) 590 – 5263.

Registration fee includes course materials, continental breakfast, refreshments, lunch and entrance to the "Meet and Greet" on Sunday evening, October 7, 2012.

"Meet and Greet"

Join us for an informal "meet and greet" on the evening of Sunday, October 7, 2012 from 6 – 7:30p.m. in the Independence Ballroom. Light refreshments will be provided. RSVP is required in advance; please check the box on the registration form.

Course Objectives

Upon completion of this activity, participants should be able to:

- Discuss developments in FA research.
- Discuss continued research, clinical trials and scientific discovery.
- Discuss the connection between disability and relationships.
- Discuss current clinical approaches to care in FA.
- Discuss the importance of advocacy.

Program

October 8, 2012

7:30 a.m.	Registration and Continental Breakfast	3 p.m.	Break
8 a.m.	Welcome and Introduction	3:20 p.m.	Accessible Travel - Panel Discussion- 45 min
8:05 a.m.	New Pathways and Therapies for FA <i>Robert Wilson, MD, Ph.D</i>	4 p.m.	Open Q&A – General Discussion Moderated by Ronald J. Bartek, President & Co-Founder FARA
8:45 a.m.	Clinical Research Advances in FA: The Findings From All Those Samples and Exams <i>David R. Lynch, MD, PhD</i>	4:45 p.m.	Adjourn
9:15 a.m.	Pregnancy in FA <i>Lisa Friedman, MSN, CRNP</i>		
9:45 a.m.	Cardiac Update <i>Kim Lin, MD</i>		
10:15 a.m.	Break		
10:30 a.m.	Overcoming Adversity <i>Jesse Billauer</i>		
Noon	Lunch		
1 p.m.	HDAC Inhibition in FA <i>James Rusche, PhD</i> <i>Sr. VP, Research and Development</i> <i>Repligen Corporation</i>		
1:30 p.m.	Gene Therapy for FA: When and How? <i>Jennifer Farmer, MS, CGC</i>		
2 p.m.	Facts, Fiction, and the Unknown: The Role of Frataxin and Mitochondrial Dysfunction in FA and How That Informs Clinical Features of FA and Therapeutic Strategies <i>Giovanni Manfredi, MD, PhD</i>		

Hotel Accommodations

A room block with reduced rates has been reserved at **Crowne Plaza Valley Forge**. The group rates are \$125 single and double occupancy plus a 8% room tax. Participants are required to make their own hotel accommodations by calling the **Crowne Plaza** directly at **610-265-7500**. The special room rate will be available until **Friday, September 7, 2012**. After that date, reservations will be accepted on a space and rate availability basis. Please mention the group name "Friedreich's Ataxia-The Children's Hospital of Philadelphia" when making your reservation.

Travel Scholarships

A limited number of travel scholarships will be offered to individuals who wish to attend the symposium and need financial assistance. For information on how to apply for a travel scholarship from the FA Project, please contact Maria at: maria@thefaproject.org. There are a limited number of scholarships available.

Cancellation and Refund Policy

The Children's Hospital of Philadelphia reserves the right to cancel or postpone any course due to any unforeseen circumstances. In the event of cancellation or postponement, The Children's Hospital of Philadelphia will refund any registration fees, less a service charge, but is not responsible for any related costs, charges or expenses to participants, including cancellation charges assessed by airlines/travel agencies.

In order to process refunds for course withdrawals, cancellations must be received in writing by September 28, 2012. No refunds will be issued thereafter. Refunds will be issued for the amount of registration minus a \$40 service charge.

Services for the Disabled

Participation by all individuals is encouraged. If arrangements are required for an individual with special needs to attend this meeting, please contact the CME Department at (215) 590 – 5263 at least two weeks in advance so that we may provide better service to you during the conference.

Organizing Committee

David R. Lynch, M.D., Ph.D.

*Attending Physician, Division of Neurology
The Children's Hospital of Philadelphia*

Kim Schadt, C.R.N.P., C.C.R.P.

*Nurse Practitioner, Trisomy 21 Program and
Friedreich Ataxia Program
The Children's Hospital of Philadelphia*

Jennifer M. Farmer, M.S., C.G.C.

*Coordinator, Collaborative Clinical Research Network in FA
Executive Director, Friedreich's Ataxia Research Alliance*

Holly L. Hedrick, M.D.

*Parent Consultant-Friedreich's Ataxia Program
The Children's Hospital of Philadelphia*

Felicia DeRosa

*Program Manager
Friedreich's Ataxia Research Alliance (FARA)*

Kyle Bryant

*Ride Ataxia, Founder/Program Director
Friedreich's Ataxia Research Alliance (FARA)*

Conference Faculty

Ronald J. Bartek

President & Co-Founder FARA

Jesse Billauer

Self-Advocate

Lisa Friedman, MSN, CRNP

Family Nurse Practitioner

Kimberly L. Lin, MD

*Attending Physician
Division of Cardiology
The Children's Hospital of Philadelphia*

Giovanni Manfredi, MD, PhD

*Professor of Neurology and Neuroscience
Weill Cornell Medical College, New York
Chief Scientific Officer, FARA*

James Rusche, PhD

*Sr. VP, Research and Development
Repligen Corporation*

Robert B. Wilson, MD, PhD

*Associate Professor of Pathology and
Laboratory Medicine
Perelman School of Medicine at the
University of Pennsylvania*

Everyday Hero

Your Everyday Heroes - Sharing Your Hero Stories

Who is your hero?

What did he or she do to win your admiration?

Maybe it was your hero's courage, outstanding achievements or noble qualities that touched you.

Share your inspiration—tell us about your hero.

Submit a brief description (50 words or less) about your hero to:

FAProgram@email.chop.edu

Join us in celebrating everyday heroes whose selfless acts, courage and stories of bravery are sure to inspire.

Fifth Annual Friedreich's Ataxia Symposium

Monday, October 8, 2012

Crowne Plaza Valley Forge, King of Prussia, PA USA

REGISTRATION FORM

Please list all attending

Name(s) _____

Home Address _____

City _____ State _____ Zip _____

Country _____ Phone _____

Fax _____ Email _____

Conference Registration Fee: \$95 per person

☐ I will attend the Meet & Greet Reception on **October 7th** _____ # of people

☐ I am a wheelchair user List of Names _____

☐ I will have a service dog _____

☐ Everyday Hero Submission (email to: FAProgram@email.chop.edu)

Method of Payment

☐ Personal Check ☐ Institutional Check ☐ Discover

☐ MasterCard ☐ Visa ☐ American Express

Credit Card # _____ Exp. Date _____

(Please make all checks payable to The Children's Hospital of Philadelphia. Registrations will not be processed unless accompanied by full payment. Faxed registrations will be accepted for credit card payment only.)

Photos/General Media:

I hereby irrevocably give The Children's Hospital of Philadelphia perpetual authorization and permission to publish in any media worldwide, any and all video and photographs taken of me without any compensation due to me. I hereby waive any right that I may have to inspect and or approve the finished product and copy that may be used in connection therewith, or the use to which it may be applied.

Mail/Fax the completed registration form and payment to:

Ms. Micah Holliday, Continuing Medical Education Department
The Children's Hospital of Philadelphia, 34th Street and Civic Center Boulevard, Philadelphia, PA 19104
Phone/215-590-5263 ~ Fax/ 215-590-4342 ~ Email/ hollidaydm@email.chop.edu