

Sixth Annual
Friedreich's Ataxia Symposium
Monday, Oct. 14, 2013



Crowne Plaza Valley Forge
King of Prussia, Pa.

www.chop.edu/cme



The Children's Hospital
of Philadelphia[®]

Hope lives here.

Symposium Information

Symposium Overview

Friedreich's Ataxia (FA) is a rare condition that leaves patients and families searching for more information and resources. While there is currently no cure for FA, efforts continue toward 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 on therapeutic approaches and current research being conducted in the field of Friedreich's Ataxia.

Symposium Objectives

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

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

Meeting Location

The symposium will be held Monday, Oct. 14, 2013, at the Crowne Plaza Valley Forge located in King of Prussia, Pa. It is situated directly across from the King of Prussia Mall, the largest shopping mall in the United States, and less than two miles from the Valley Forge National Historic Park, the site of Washington's troops' winter encampment during the Revolutionary war.

Hotel Accommodations

A room block with reduced rates has been reserved at the Crowne Plaza Valley Forge. The group rate is \$125 single and double occupancy plus 8 percent room tax. Participants are required to make their own hotel reservations by calling the Crowne Plaza directly at 610-265-7500. The special room rate will be available until Wednesday, Sept. 13, 2013. After that date, reservations will be accepted on a space and rate availability basis. Please mention "Friedreich's Ataxia – The Children's Hospital of Philadelphia" when making your reservation.

Meet-and-Greet

Join us for an informal meet-and-greet on **Sunday, Oct. 13, 2013**, from 6 to 7:30 p.m., in the Independence Ballroom. Light refreshments will be provided. Your R.S.V.P. is required in advance; please check the box on the registration form.

Travel Scholarship

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

Symposium Information *continued*

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 Hospital will refund registration fees, less a \$40 service fee, but is not responsible for any related costs, charges or expenses to participants, including cancellation charges assessed by airlines or travel agencies. In order to process refunds, cancellations must be received in writing by Friday, Oct. 4, 2013. No refunds will be issued thereafter.

Services for People with Disabilities

If special arrangements are required for an individual with a disability to attend this meeting, please contact the Continuing Medical Education Department at 215-590-5263.

Everyday Hero

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!



Program • Monday, Oct. 14, 2013

- 7:30 a.m. **Registration and Continental Breakfast**
- 8 a.m. **Welcome and Introduction**
David R. Lynch, M.D., Ph.D.
- 8:05 a.m. **Neurological Selectivity and Restoration in FA**
David R. Lynch, M.D., Ph.D.
- 8:45 a.m. **Presymptomatic Testing and Diagnosis of FA**
Jennifer Farmer, M.S., C.G.C.
Karla W. Brigatti, M.S., C.G.C.
- 9:15 a.m. **Gene Therapy Approaches**
- 9:45 a.m. **Interferon Gamma and Ubiquitin Competitors: Novel Approaches to Raising Frataxin**
Roberto Testi, M.D.
- 10:15 a.m. **Break**
- 10:30 a.m. **Ataxia Athletes**
Kyle Bryant
- 11 a.m. **Physical Activity and Mobility**
Joyce R. Maring, P.T., D.P.T., Ed.D.
- 11:30 a.m. **FA and My Heart**
Subha Raman, M.D.
- Noon** **Lunch**
- 1:15 p.m. **Clinical Trial Refresher**
Jennifer Farmer, M.S., C.G.C.
- 1:45 p.m. **Molecular Mechanisms in FA: New Developments**
Robert B. Wilson, M.D., Ph.D.
- 2:15 p.m. **Break**
- 2:30 p.m. **Patient Panel: Driving and Adaptions**
Dynah Haubert
- 3 p.m. **Open Q&A – General Discussion**
Moderated by Ronald J. Bartek
- 4 p.m. **Adjourn**

Symposium Faculty

Organizing Committee

David R. Lynch, M.D., Ph.D.
Director, Friedreich's Ataxia Program
Attending Physician
Division of Neurology
The Children's Hospital of Philadelphia

Kim Schadt, M.S.N., C.R.N.P., C.C.R.P.
Nurse Practitioner
Trisomy 21 and Friedreich's Ataxia Programs
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 (FARA)

Holly Hedrick, M.D., F.A.C.S.
Parent Consultant
Friedreich's Ataxia Program
The Children's Hospital of Philadelphia

Felicia DeRosa, M.P.A.
Program Director
FARA

Kyle Bryant
Ride Ataxia
Founder/Program Director
FARA

Symposium Faculty

Karlla W. Brigatti, M.S., C.G.C.
Friedreich's Ataxia Research Program
Genetic Counselor, Neurogenetics
The Children's Hospital of Philadelphia

Ronald J. Bartek
President & Co-Founder
FARA

Dynah Haubert
Patient Advocate

Joyce R. Maring, P.T., D.P.T., Ed.D.
Associate Professor
Program Director
Doctor of Physical Therapy Program
Chair, Physical Therapy and
Healthcare Sciences
The George Washington University

Subha Raman, M.D.
Professor and Associate Director for Quality
Division of Cardiovascular Medicine
The Ohio State University Medical Center

Roberto Testi, M.D.
Chair of Immunology
Department of Biomedicine and Prevention
Rare Disease Section
School of Medicine
University of Rome "Tor Vergata"

Robert B. Wilson, M.D., Ph.D.
Professor of Pathology and Laboratory Medicine
Perelman School of Medicine at the
University of Pennsylvania

Registration

Friedreich's Ataxia Symposium

Monday, Oct. 14, 2013 – *Registration Deadline: Monday, Sept. 30*

Name _____

Home Address _____

City _____ State _____ ZIP _____

Phone _____

Email _____

Others attending with you: _____

I will attend the Meet-and-Greet Reception on Sunday, Oct. 13 with _____ people.

I am a wheelchair user I have a service dog

Everyday Hero submission (email to FAProgram@email.chop.edu)

Symposium Fee: \$95 per person

Registration will not be processed unless accompanied by full payment.

Faxed registrations will be accepted for credit card payments only.

Method of Payment

Personal check Institutional check

(Please make check payable to The Children's Hospital of Philadelphia.)

Discover MasterCard American Express VISA

Credit Card # _____

Exp. Date _____

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 19104-4399 • 215-590-5CME • Fax 215-590-4342 •
hollidaydm@email.chop.edu

Photos/General Media:

I hereby irrevocably give The Children's Hospital of Philadelphia perpetual authorization and permission to publish in any media, any and all video and photograph taken of me at the CHOP Friedreich's Ataxia Symposium 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 conjunction therewith, or the use to which it may be applied.

Signature: _____

Register online at www.chop.edu/cme