

Ninth Annual **Friedreich's Ataxia Symposium**

Monday, Oct. 17, 2016



**Crowne Plaza
Valley Forge**
King of Prussia, Pa.

www.chop.edu/cme

Friedreich's Ataxia Center of Excellence

 **The Children's Hospital of Philadelphia®**
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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 Center of Excellence 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. 17, 2016, at the Crowne Plaza Valley Forge in King of Prussia, Pa. The hotel is directly across from the King of Prussia Mall, the largest shopping mall in the United States, and is 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 block of rooms with reduced rates has been reserved at the Crowne Plaza Valley Forge. The group rate is \$129 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 Tuesday, Sept. 13, 2016. 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. 16 from 6 to 7:30 p.m. Light refreshments will be provided. **Your RSVP is required in advance; please check the box on the registration form.**

Poster Presentation

A special poster presentation will showcase current research, insights and innovative strategies in Friedreich's ataxia by young investigators, researchers and postdoctoral students. This will provide the opportunity for the research community to interact with patients and families while simultaneously allowing families to further engage the scientific community. An interactive poster session with the presenters will be featured on Sunday evening during the meet-and-greet reception. Posters can also be viewed during the lunch break on Monday.

Symposium Information continued

Travel Scholarships

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. The FA Project is a nonprofit organization created to raise awareness and funds to benefit those affected by Friedreich's ataxia. There is a limited number of scholarships available. **The deadline to submit is Friday, Sept. 16, 2016.**

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 Monday, Oct. 3, 2016. 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. A two-week notice is preferred.

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. 17, 2016

- 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.** **Sensory Dysfunction in Friedreich's Ataxia**
David R. Lynch, M.D., Ph.D.
- 8:30 a.m.** **Effects of Symptomatic Care in Friedreich's Ataxia:
Impact on Disease Evolution with Standardized Follow-up**
Isabelle Husson, M.D., Ph.D.
- 9:15 a.m.** **Exercise in Friedreich's Ataxia: Panel Discussion**
Kimberly Y. Lin, M.D.; Shana McCormack, M.D., M.T.R.; Bastien Roche, P.T.
- 10:15 a.m.** **Break**
- 10:30 a.m.** **FARA Strategies and FA Treatment Pipeline**
Jennifer Farmer, M.S., C.G.C.
- 11 a.m.** **Epigenetics of Friedreich's Ataxia**
Sanjay I. Bidichandani, M.B.B.S., Ph.D.
- Noon** **Lunch**
- 1:15 p.m.** **Clinical Trials in Friedreich's Ataxia: Current Status**
David R. Lynch, M.D., Ph.D.
- 2 p.m.** **Metabolic Signaling in Heart as a Marker of Disease**
R. Mark Payne, M.D., F.A.A.P., F.A.C.C.
- 2:45 p.m.** **Break**
- 3:10 p.m.** **Patient Panel: Exercise**
Moderator: Kyle Bryant
- 3:40 p.m.** **Open Q&A — General Questions**
- 4 p.m.** **Adjourn**

Symposium Faculty

Unless otherwise noted, faculty is from The Children's Hospital of Philadelphia and the Perelman School of Medicine at the University of Pennsylvania.

Organizing Committee

Kyle Bryant

rideATAXIA

Founder/Program Director

FARA

Felicia DeRosa, M.P.A.

Program Director

FARA

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

Coordinator

Collaborative Clinical Research Network in FA

Executive Director

Friedreich's Ataxia Research Alliance (FARA)

Holly L. Hedrick, M.D., F.A.C.S.

Parent Consultant

Friedreich's Ataxia Center of Excellence

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

Co-director

Friedreich's Ataxia Center of Excellence

Attending Physician

Division of Neurology

Professor of Neurology

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

Nurse Practitioner

Friedreich's Ataxia Center of Excellence

Symposium Faculty

Sanjay I. Bidichandani, M.B.B.S., Ph.D.

CMRI Claire Gordon Duncan Chair in Genetics

Professor of Pediatrics

University of Oklahoma College of Medicine

Oklahoma City, Okla.

Isabelle Husson, M.D., Ph.D.

Service de Neurologie Pédiatrique et des

Maladies Métaboliques

INSERM U676

Hôpital Robert Debré – AP-HP

Paris, France

Kimberly Y. Lin, M.D.

Assistant Professor

Division of Cardiology

Penn-CHOP Friedreich's Ataxia Center of

Excellence, Cardiac Program Director

Shana McCormack, M.D., M.T.R.

Attending Physician

Division of Endocrinology and Diabetes

Assistant Professor of Pediatrics

R. Mark Payne, M.D., F.A.A.P., F.A.C.C.

Professor of Pediatrics (Cardiology)

Indiana University School of Medicine

Indianapolis, Ind.

Bastien Roche, P.T.

Physiotherapist in Child Neurology

Hôpital Robert Debré – AP-HP

Paris, France

Registration – Friedreich’s Ataxia Symposium

Monday, Oct. 17, 2016 — Registration deadline: Friday, Sept. 30, 2016

* Name _____

Home Address _____

City _____ State _____ ZIP _____

Phone _____

* Email _____

(*Required field)

Conference Registration Fee: \$95 per person (includes conference registration, materials, breakfast, lunch and breaks • maximum charge per family \$285)

I will attend the conference.

Others attending conference: _____
(Please list all) _____

I will attend the Meet-and-Greet Reception on Sunday, Oct. 16 with _____ additional people.

I am a first-time conference attendee.

I am a wheelchair user. I have a service dog.

I have a personal care assistant (PCA) attending with me. (Name: _____)

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

Method of Payment

Total \$ _____

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 • 3401 Civic Center Blvd. • Philadelphia, Pa. 19104-4399 • 215-590-5CME • Fax 215-590-4342 • hollidaydm@email.chop.edu

Cover photo courtesy of Positive Exposure

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 photographs 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