

Eighth Annual **Friedreich's Ataxia Symposium**



Monday, Oct. 12, 2015

**DoubleTree by Hilton Hotel
Philadelphia – Valley Forge**
King of Prussia, Pa.

www.chop.edu/cme

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. 12, 2015, at the DoubleTree by Hilton Hotel Philadelphia – Valley Forge. Located just 30 minutes from Philadelphia International Airport, the hotel is situated on nine acres of beautifully landscaped grounds in King of Prussia, Pa. Travelers interested in shopping and dining will appreciate our Valley Forge hotel's proximity to the King of Prussia Mall as well as fantastic array of dining establishments. For those interested in history, take advantage of our hotel's proximity to Valley Forge National Park and explore historical artifacts and preserved sites from revolutionary war encampments. In addition, guests can take a trip through Longwood Gardens, or take in some culture and history by visiting the Barnes Foundation and Winterthur Museum.

301 West DeKalb Pike, King of Prussia, Pa. 19406 • 610-337-1200

<http://doubletree3.hilton.com/en/hotels/pennsylvania/doubletree-by-hilton-hotel-philadelphia-valley-forge-PHLVFDT/index.html>

Hotel Accommodations

A room block with reduced rates has been reserved at the DoubleTree by Hilton Hotel Philadelphia – Valley Forge. The group rate is \$99 single and double occupancy plus applicable taxes. Participants are required to make their own reservations by calling the hotel directly at 610-337-1200 or by contacting the on-site reservation contact, Mindy Toussaint, at 484-684-1421 or Mindy.Toussaint@hilton.com. Please mention “**Friedreich's Ataxia Symposium – The Children's Hospital of Philadelphia**” when making your reservation. The special room rate will be available until Sunday, Sept. 27, 2015. After that date, reservations will be accepted on a space and rate availability basis..

Please Note: There are a limited number of accessible rooms at the DoubleTree. Please call the hotel directly. Rooms are on a first-come, first-served basis.

Meet-and-Greet

Join us for an informal meet-and-greet on Sunday, Oct. 11 from 6 to 7:30 p.m. Light refreshments will be provided. Your R.S.V.P. is required in advance; please check the box on the registration form.

Symposium Information *continued*

Poster Presentation

A special poster presentation will showcase current research, insights and innovative strategies in Friedreich's ataxia by young investigators, researchers and post-doctoral 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.

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. The FA Project is a nonprofit organization created to raise awareness and funds to benefit those affected by Friedreich's ataxia. 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 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 Saturday, Oct. 3, 2015. 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. 12, 2015

- 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. Understanding the Neurologic Dysfunction and Clinical Management in Friedreich's Ataxia (FA)
David R. Lynch, M.D., Ph.D.
- 8:45 a.m. Natural History, Clinical Outcomes and Patient Registry
Jen Farmer, M.S., C.G.C.
- 9:15 a.m. FA Drug Discovery and Treatment Pipeline
Robert B. Wilson, M.D., Ph.D.
- 9:45 a.m. A View Through the Microscope to the Mitochondria in Neurons in Models of FA
Jordi Magrane, Ph.D.
- 10:15 a.m. Break and Posters
- 10:30 a.m. MOXIe Trial (RTA-408)
Colin J. Meyer, M.D.
- 10:50 a.m. STEADFAST Trial (ACTIMMUNE)
Julie Ball
- 11:10 a.m. dPFUFAs and RT0001 Trial
Harry J. Saal, Ph.D.
- 11:30 a.m. Q&A
- 12 p.m. Lunch
Rick Guidotti, Positive Exposure
- 1:15 p.m. Cardiac Function and Exercise in FA
Kimberly L. Lin, M.D.
- 1:45 p.m. Gene Therapy Approaches for FA: Research in the FA Mice
Hélène Puccio, Ph.D.
- 2:15 p.m. Muscle Spectroscopy in FA
Shana E. McCormack, M.D.
- 2:45 p.m. Break
- 3 p.m. FARA Ambassadors: Decision-making, Accessibility and Resources for College
Moderator: Norman Simpson
- 3:30 p.m. Open Q&A – General Questions
Moderator: Ronald J. Bartek
- 4 p.m. Adjourn

Symposium Faculty

Organizing Committee

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

Co-director, Friedreich's Ataxia Center of Excellence

Attending Physician

Division of Neurology

The Children's Hospital of Philadelphia

Professor of Neurology

Perelman School of Medicine at the University of Pennsylvania

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

Nurse Practitioner

Friedreich's Ataxia Center of Excellence

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

Parent Consultant

Friedreich's Ataxia Center of Excellence

The Children's Hospital of Philadelphia

Felicia DeRosa, M.P.A.

Program Director

FARA

Kyle Bryant

Ride Ataxia

Founder/Program Director

FARA

Symposium Faculty

Julie Ball

Senior Director, Clinical Operations

Horizon Pharma USA, Inc.

Ronald J. Bartek

President and Co-founder

FARA

Rick Guidotti

Positive Exposure

Kimberly Y. Lin, M.D.

Attending Cardiologist, Cardiac Center

The Children's Hospital of Philadelphia

Jordi Magrane, Ph.D.

Assistant Professor of Neuroscience

Weill Cornell Medical School

Shana E. McCormack, M.D.

Attending Physician

The Diabetes Center for Children and the

Congenital Hyperinsulinism Center

The Children's Hospital of Philadelphia

Colin J. Meyer, M.D.

Vice President, Product Development

Chief Medical Officer

Reata Pharmaceuticals, Inc.

Hélène Puccio, Ph.D.

Research Director

INSERM

Harry J. Saal, Ph.D.

Chairman

Retrotape Inc.

Norman Simpson

Parent Advocate

Robert B. Wilson, M.D., Ph.D.

Co-director, Friedreich's Ataxia Center of Excellence

Professor of Pathology and Laboratory Medicine

Perelman School of Medicine at the

University of Pennsylvania

Registration – Friedreich’s Ataxia Symposium

Monday, Oct. 12, 2015 — Registration deadline: Monday, Sept. 21, 2015

* Name _____

Home Address _____

City _____ State _____ ZIP _____

Phone _____

* Email _____

*(*Required field)*

Conference Registration Fee: \$95 per person *(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. 11 with _____ 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.

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

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