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

The Children’s Hospital of Philadelphia®
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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

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!
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

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
Retrotope 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

Symposium Faculty

Julie Ball
Senior Director, Clinical Operations
Horizon Pharma USA, Inc.

Ronald J. Bartek
President and Co-founder
FARA
Registration – Friedreich’s Ataxia Symposium


* 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: _________________________________________________________________