

10th Annual Friedreich's Ataxia Symposium

Monday, Oct. 16, 2017

Crowne Plaza Valley Forge
King of Prussia, PA

Learn more:
chop.cloud-cme.com



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 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

Registration Fee

The registration fee is \$95 per person. This fee includes conference registration materials, breakfast, lunch, breaks and welcome reception. To register, please visit **chop.cloud-cme.com**.

Meeting Location

The symposium will be held Monday, Oct. 16, 2017, 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 Wednesday, Sept. 27, 2017. After that date, reservations will be accepted on a space and rate availability basis. Please mention the name of this conference when making your reservation.

Meet-and-Greet

Join us for an informal meet-and-greet on Sunday, Oct. 15 from 6 to 7:30 p.m. at the hotel. Light refreshments will be provided. **Your RSVP 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 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.

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. **The deadline to submit is Friday, Sept. 8, 2017.**

Cancellation and Refund Policy

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. 2, 2017. 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. 16, 2017

- 7:30 a.m. **Registration and Continental Breakfast**
- 8 a.m. **Welcome and Introduction**
David R. Lynch, MD, PhD
- 8:05 a.m. **FRDA: from basic science to clinical features**
David R. Lynch, MD, PhD
- 8:45 a.m. **FARA programs and FRDA treatment pipeline**
Jennifer M. Farmer, MS, CGC
- 9:15 a.m. **Identification of p38 MAPK as a novel therapeutic target for FRDA**
Robert B. Wilson, MD, PhD
- 9:45 a.m. **The Future of Fashion: Mainstream Adaptive Clothing**
Mindy Scheier
- 10:15 a.m. **Break**
- 10:30 a.m. **Center of Excellence Research Update**
Ian A. Blair, PhD
Kimberly Y. Lin, MD
Shana McCormick, MD, MTR
- 11:30 a.m. **Q&A**
- 12 p.m. **Lunch**
- 1:15 p.m. **Activating Frataxin Expression with Synthetic Oligonucleotides**
David Corey, PhD
- 1:45 p.m. **Developing Gene Delivery Vehicles for Widespread Frataxin Expression in Brain and Beyond**
Ben Deverman, PhD
- 2:15 p.m. **Q&A**
- 2:45 p.m. **Break**
- 3 p.m. **Global FRDA Community and RideATAXIA Europe**
Anabella Saraiva
Kyle Bryant
Robert B. Wilson, MD, PhD
- 3:30 p.m. **Open Q&A – General Questions**
- 4 p.m. **Adjourn**

Symposium Faculty

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

Organizing Committee

Kyle Bryant

rideATAXIA

Founder/Program Director

Friedreich's Ataxia Research Alliance (FARA)

Felicia DeRosa, MPA

Program Director

FARA

Jennifer M. Farmer, MS, CGC

Coordinator

Collaborative Clinical Research Network in FA

Executive Director

FARA

Holly L. Hedrick, MD, FACS

Parent Consultant

Friedreich's Ataxia Center of Excellence

David R. Lynch, MD, PhD

Co-director

Friedreich's Ataxia Center of Excellence

Attending Physician

Division of Neurology

Professor of Neurology

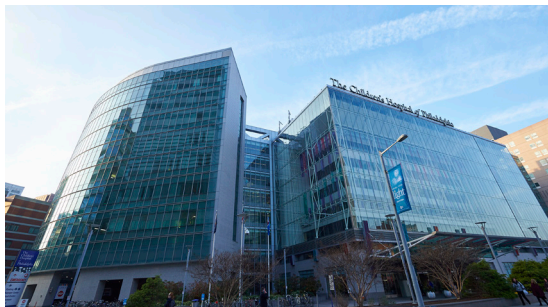
Kim Schadt, MSN, CRNP

Nurse Practitioner

Friedreich's Ataxia

Center of Excellence

continued >



Symposium Faculty continued

Symposium Faculty

Ian A. Blair, PhD

A.N. Richards Professor

Vice-Chair, Department of Systems Pharmacology and Translational Therapeutics

Director, Penn Superfund Research and Training Program Center

University of Pennsylvania

David Corey, PhD

Rusty Kelley Professor of Medical Sciences

Departments of Pharmacology and Biochemistry

UT Southwestern Medical Center

Dallas, Texas

Ben Deverman, PhD

Director, Beckman Institute CLARITY, Optogenetics and Vector Engineering Research Center (CLOVER Center)

California Institute of Technology

Kimberly Y. Lin, M.D.

Attending Cardiologist

Assistant Professor

Section of Cardiomyopathy and Heart Transplantation

Division of Cardiology

Shana McCormack, MD, MTR

Attending Physician

Division of Endocrinology and Diabetes

Assistant Professor of Pediatrics

Anabella Saraiva

Member French Patient Advocacy Group – Francaise de l'Ataxia de Friedreich (AFAF)

Coordinator, Ride Ataxia France

Mindy Scheier

Founder and President, Runway of Dreams

Robert B. Wilson, MD, PhD

Senior Scientist, Center for Mitochondrial and Epigenomic Medicine

Professor, Department of Pathology and Laboratory Medicine