

11th Annual Friedreich's Ataxia Symposium

Monday, Oct. 15, 2018

Crowne Plaza Valley Forge
King of Prussia, PA

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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 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. 15, 2018, 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 \$132 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 Friday, Sept. 21, 2018. 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. 14, 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.**

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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 **first-time attendees** 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. 7, 2018.**

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. 1, 2018. 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 fewer) 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. 15, 2018

- 7:30 a.m. Registration and Continental Breakfast
- 8 a.m. Welcome and Introduction
David R. Lynch, MD, PhD
- 8:05 a.m. Novel Alliterative Therapies: Statins, Steroids and Stem Cells – Fact, Fiction or Friction
David R. Lynch, MD, PhD
- 8:45 a.m. Clinical Trial and Pipeline Status Updates
Jennifer Farmer, MS, CGC
- 9:15 a.m. Novel Targets for Drug Discovery in Friedreich Ataxia
Robert B. Wilson, MD, PhD
- 9:45 a.m. Break
- 10 a.m. Can Frataxin Expression be Restored in Friedreich's Ataxia Using Drugs that Modify Chromatin Structure?
David Jacoby, MD, PhD
- 10:30 a.m. Update from Chondrial: Frataxin Replacement Therapy
Carole S. Ben-Maimon, MD
- 11:30 a.m. Q&A
- Noon Lunch
- 1:15 p.m. FXN Gene Activation Panel
Sanjay I. Bidichandani, MBBS, PhD
Aseem Z. Ansari, BSc, PhD
Manuela Corti, PT, PhD
- 2:15 p.m. Cardiac and Metabolism Panel
Kimberly Y. Lin, MD
Shana McCormick, MD, MTR
- 2:45 p.m. Break
- 3 p.m. Patient Panel
Moderated by Kyle Bryant
- 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

Symposium Faculty

Aseem Z. Ansari, BSc, PhD

Professor

University of Wisconsin-Madison

Carole S. Ben-Maimon, MD

CEO, Chondrial Therapeutics

Sanjay I. Bidichandani, MBBS, PhD

CMRI Claire Gordon Duncan Chair in Genetics

Professor of Pediatrics

University of Oklahoma College of Medicine

Manuela Corti, PT, PhD

Assistant Professor

University of Florida

David Jacoby, MD, PhD

Vice President, Head of Clinical Sciences

BioMarin Pharmaceuticals

Kimberly Y. Lin, MD

Friedreich Ataxia Center of Excellence Cardiac

Program Director

Assistant Professor, Division of Cardiology

Section of Cardiomyopathy and Heart

Transplantation

Shana McCormack, MD, MTR

Attending Physician, Division of Endocrinology

and Diabetes

Assistant Professor of Pediatrics

Robert B. Wilson, MD, PhD

Senior Scientist, Center for Mitochondrial and

Epigenomic Medicine

Professor, Department of Pathology and

Laboratory Medicine