



# **Fourth Annual Friedreich's Ataxia Symposium**

**October 8, 2011**

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

**Sponsored by the Friedreich's Ataxia Program at**

**CH The Children's Hospital of Philadelphia®**

**Hope lives here.**

## **Symposium Overview**

Worldwide, Friedreich's Ataxia (FA) affects roughly one in 50,000 people, making it the most common in a group of related disorders called hereditary ataxias. While there is currently no cure for FA, efforts continue to be made towards research that will one day halt or even reverse disease progression. The Friedreich's Ataxia Program 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, therapeutic approaches and current research being conducted in the field of Friedreich's Ataxia.

## **Meeting Location**

The symposium will be held Saturday, October 8, 2011 at the Crowne Plaza Valley Forge located in King of Prussia, Pa. Perfectly situated directly across from the King of Prussia Mall, the largest shopping mall in the United States. Valley Forge National Historic Park, the site of Washington's troops winter encampment during the Revolutionary war is also located less than two miles away.

## **Course 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.
- Discuss the connection between disability and relationships.

## **Registration Information**

Pre-registration is required and the registration deadline is September 23, 2011. Payment must accompany registration form for it to be processed. Once registration has been processed a confirmation letter will be mailed or faxed. PLEASE NOTE: Registration is not complete if you do not receive this confirmation. If you do not receive a confirmation within 10 days of registration, please contact us at (215) 590 – 5263.

Registration fee includes course materials, continental breakfast, refreshments, lunch and entrance to the "Meet and Greet" on Friday evening, October 7, 2011.

## **"Meet and Greet"**

Join us for an informal "meet and greet" on the evening of Friday, October 7, 2011 from 6 – 7:30p.m. in the Independence Ballroom. Light refreshments will be provided. RSVP is required in advance; please check the box on the registration form.

**[www.chop.edu/cme](http://www.chop.edu/cme)**

## Hotel Accommodations

A room block with reduced rates has been reserved at **Crowne Plaza Valley Forge**. The group rates are \$119 single and double occupancy plus a 8% room tax. Participants are required to make their own hotel accommodations by calling the **Crowne Plaza** directly at **610-265-7500**. The special room rate will be available until Wednesday, September 7, 2011. After that date, reservations will be accepted on a space and rate availability basis. Please mention the group name "Friedreich's Ataxia-The Children's Hospital of Philadelphia" when making your reservation.

## Travel Scholarships

A limited number of travel scholarships will be offered to individuals who wish to attend the symposium and need financial assistance. For information on how to apply for a travel scholarship from the FA Project, please contact Maria at: [maria@thefaproject.org](mailto:maria@thefaproject.org). 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 Children's Hospital of Philadelphia will refund any registration fees, less a service charge, but is not responsible for any related costs, charges or expenses to participants, including cancellation charges assessed by airlines/travel agencies.

In order to process refunds for course withdrawals, cancellations must be received in writing by September 29, 2011. No refunds will be issued thereafter. Refunds will be issued for the amount of registration minus a \$40 service charge.

## Services for the Disabled

Participation by all individuals is encouraged. If arrangements are required for an individual with special needs to attend this meeting, please contact the CME Department at (215) 590 – 5263 at least two weeks in advance so that we may provide better service to you during the conference.

## Organizing Committee

**David R. Lynch, M.D., Ph.D.**  
Attending Physician, Division of Neurology  
The Children's Hospital of Philadelphia

**Kim Schadt, C.R.N.P., C.C.R.P.**  
Nurse Practitioner, Trisomy 21 Program and  
Friedreich Ataxia Program  
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

**Holly L. Hedrick, M.D.**  
Parent Consultant-Friedreich's Ataxia Program  
The Children's Hospital of Philadelphia

**Felicia DeRosa**  
Program Manager  
Friedreich's Ataxia Research Alliance (FARA)

**Kyle Bryant**  
Ride Ataxia, Founder/Program Director  
Friedreich's Ataxia Research Alliance (FARA)

## Conference Faculty

**Gabrielle Angiolelli**  
Patient Advocate

**Roger Crawford**  
Roger Crawford & Associates

**Allison Dana**  
Patient Advocate

**Mirella Dottori, Ph.D.**  
Centre for Neuroscience and Department of Pharmacology  
The University of Melbourne, Australia

**Joel M. Gottesfeld, Ph.D.**  
Professor  
Department of Molecular Biology  
The Scripps Research Institute

**Donna Littell**  
Patient Advocate

**Marek Napierala, Ph.D., M.D.**  
Assistant Professor, Department of Biochemistry  
Anderson Cancer Center

**Massimo Pandolfo, M.D.**  
Professor, Neurology  
Hôpital Erasme - Université Libre de Bruxelles, Belgium

**Jade Perry**  
Patient Advocate

**Hélène Puccio, Ph.D.**  
Research Director INSERM  
Department of Neurobiology and Genetics  
Institut de Genetique et de Biologie Moléculaire et Cellulaire (IGBMC), France

**Steven M. Willi, M.D.**  
Director, Diabetes Center for Children  
Division of Endocrinology  
The Children's Hospital of Philadelphia

**Robert B. Wilson, M.D., Ph.D.**  
Associate Professor of Pathology and Laboratory Medicine  
University of Pennsylvania School of Medicine

## Everyday Hero

Your Everyday Heroes - Sharing Your Hero Stories

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](mailto:FAProgram@email.chop.edu)

Join us in celebrating everyday heroes whose selfless acts, courage and stories of bravery are sure to inspire.

## October 8, 2011

7:30 a.m.	<b>Registration and Continental Breakfast</b>
8 a.m.	<b>Welcome and Introduction</b>
8:05 a.m.	<b>Neurological Features of Friedreich's Ataxia</b> <i>David R. Lynch, M.D., Ph.D.</i>
8:45 a.m.	<b>Diabetes in Friedreich's Ataxia</b> <i>Steven M. Willi, M.D.</i>
9:15 a.m.	<b>Biochemical Mechanisms and Therapeutic Pathways</b> <i>Massimo Pandolfo, M.D. and Robert Wilson, M.D.</i>
10 a.m.	<b>Break and Exhibits</b>
10:30 a.m.	<b>Turning the Pessimism of "I Can't" Into the Unstoppable Power of "I Can!"</b> <i>Roger Crawford</i>
Noon	<b>Lunch and Exhibits</b>
1 p.m.	<b>Q &amp; A Session – Clinical Panel</b> – Several physicians and clinical care providers from various specialty areas (neurology, cardiology, genetics and endocrinology) will participate in a moderated open Q&A session where symposium participants can ask questions about FA symptoms, diagnosis and treatment.
1:45 p.m.	<b>Research Topics and Discussion</b> – Several FA researchers will present brief updates (15 minutes) on their work and how it relates to furthering our understanding of FA and the discovery and advancement of treatments. <i>Mirella Dottori, Ph.D., Joel Gottesfeld, Ph.D., Marek Napierala, Ph.D. and Hélène Puccio, Ph.D.</i>
2:45 p.m.	<b>Q &amp; A Session – Research Panel</b> – All of the researchers who participated in previous sessions will participate in a moderated open Q&A session where symposium participants can ask questions about the FA gene, frataxin function, mechanisms of the disease, animal and cellular models, drug discovery and development research.
3:30 p.m.	<b>Break and Exhibits</b>
4 p.m.	<b>Canine Assistant Panel</b> <i>Gabrielle Angiolelli</i> <i>Allison Dana</i> <i>Jade Perry</i> <i>Donna Littell</i>
5 p.m.	<b>Symposium Adjourns</b>

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# REGISTRATION FORM

Name(s) \_\_\_\_\_

\_\_\_\_\_

Home Address \_\_\_\_\_

City \_\_\_\_\_ State \_\_\_\_\_ Zip \_\_\_\_\_

Country \_\_\_\_\_ Phone \_\_\_\_\_

Fax \_\_\_\_\_ Email \_\_\_\_\_

**Conference Registration Fee: \$95 per person**

- ☐ I will attend the Meet & Greet Reception on **October 7th** \_\_\_\_\_ # of people
- ☐ I am a wheelchair user
- ☐ I will have a service dog
- ☐ Everyday Hero Submission (email to: [FAProgram@email.chop.edu](mailto:FAProgram@email.chop.edu))

**Method of Payment**

- ☐ Personal Check      ☐ Institutional Check      ☐ Discover  
☐ MasterCard      ☐ Visa      ☐ American Express

Credit Card # \_\_\_\_\_ Exp. Date \_\_\_\_\_

*(Please make all checks payable to The Children's Hospital of Philadelphia. Registrations will not be processed unless accompanied by full payment. Faxed registrations will be accepted for credit card payment only.)*

Photos/General Media:

I hereby irrevocably give The Children's Hospital of Philadelphia perpetual authorization and permission to publish in any media worldwide, any and all video and photographs taken of me 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 connection therewith, or the use to which it may be applied.

**Mail/Fax the completed registration form and payment to:**

Ms. Micah Holliday, Continuing Medical Education Department  
The Children's Hospital of Philadelphia, 34<sup>th</sup> Street and Civic Center Boulevard, Philadelphia, PA 19104  
**Phone/215-590-5263 ~ Fax/ 215-590-4342 ~ Email/ [hollidaydm@email.chop.edu](mailto:hollidaydm@email.chop.edu)**