



BP1 Disorders

Leading the Charge For a Cure

SATURDAY, JUNE 22 - STXBP1 INVESTIGATORS & FAMILY MEETING - SCIENCE + LOVE = CURE

3549 Chestnut St., Sheraton University City, Philadelphia

8:00 - 8:25 AM	Continental Breakfast & Featured Family Stories – Hioco & Clatterbuck Stories
8:25 - 8:30 AM	Kick-off & Rules – Anne Johnson; STXBP1 Foundation
8:30 - 12:00 PM	Morning Session
8:30 - 8:55 AM	<ul style="list-style-type: none">I. Helbig, MD; <i>The Children's Hospital of Philadelphia</i> – Presentation, Prognosis, Misconceptions, & Therapies on the Horizon for STXBP1 Disorders
8:55 - 9:20 AM	<ul style="list-style-type: none">A. Goldstein, MD; <i>The Children's Hospital of Philadelphia</i> – Secondary Mitochondrial Conditions in STXBP1 Disorders
9:20 - 9:45 AM	<ul style="list-style-type: none">A. Field, MOTR/L; H. Milligan, PT, MPT; <i>The Children's Hospital of Philadelphia</i> – Physical & Occupational Therapy Experience with STXBP1 & Similar Conditions
9:45 - 10:00 AM	<ul style="list-style-type: none">Panel – Questions
10:00 - 10:10 AM	BREAK
10:10 - 10:35 AM	<ul style="list-style-type: none">C. Hopkins, PhD, MBA; <i>NemaMetrix</i> – Using Worms to Study Changes in the STXBP1 Gene
10:35 - 11:00 AM	<ul style="list-style-type: none">W. Chen, PhD; <i>Baylor College of Medicine</i> – Understanding STXBP1 Disorders in Mouse Models
11:00 - 11:25 AM	<ul style="list-style-type: none">H. Lammertse; <i>VU University of Amsterdam</i> – Studying Consequences of STXBP1 Mutations
11:25 - 11:40 PM	<ul style="list-style-type: none">Panel – Questions
11:40 - 11:50 PM	BREAK
11:50 - 3:15 PM	Lunch & Afternoon Presentations
11:50 - 12:15 PM	<ul style="list-style-type: none">K. Helbig, MS, LCGC; <i>The Children's Hospital of Philadelphia</i> – STXBP1 & Genetic Reports
12:15 - 12:40 PM	<ul style="list-style-type: none">J. Bain, MD, PhD; <i>Simons Searchlight</i> – Understanding Registries & What They Can Tell Us
12:40 - 12:50 PM	<ul style="list-style-type: none">Panel Questions
12:50 - 1:20 PM	<ul style="list-style-type: none">L. Lubbers, PhD; <i>CURE</i> – Brainstorm: Supporting the STXBP1 Community
1:20 - 1:30PM	BREAK
1:30 - 1:55 PM	<ul style="list-style-type: none">A. Crawford, PhD; <i>Institute for Orphan Drug Discovery</i> – Fishing for Drugs
1:55 - 2:25 PM	<ul style="list-style-type: none">D. Abranov, N. Guiberson & W. Parker, MD, PhD, Z. Grinspan MD; <i>Weill Cornell Medical College</i> – Studying Drugs on STXBP1 Patient Cells
2:25 - 2:55 PM	<ul style="list-style-type: none">J. Oldenhof, PhD; <i>BioPharma</i> – How a Drug is Developed, Tested, & Approved
2:55 - 3:05 PM	<ul style="list-style-type: none">Panel – Questions
3:05 - 3:15 PM	BREAK
3:15 - 5:00 PM	Breakout Sessions / Family Discussion – M. Grant; MG Advisory
3:15 - 3:50 PM	<ul style="list-style-type: none">Purpose, Goals & Intent of Today's Discussion
3:50 - 4:40 PM	<ul style="list-style-type: none">Family Discussions & Reporting
4:40 - 5:00 PM	<ul style="list-style-type: none">The Patient Voice & Attitudes Towards Research



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FRIDAY, JUNE 21 - STXBP1 INVESTIGATORS & FAMILY MEETING - SCIENCE + LOVE = CURE

3549 Chestnut St., Sheraton University City, Philadelphia

8:00 AM - 6:00 PM

Simons Registry & Blood Draws – William Penn I & II

Participate in the Simons natural history study (disease progression) by providing blood samples and by answering registry questions.

Also available:

- Saturday 8:30 AM – 2:00 PM
Intermittently set during the breaks in talks, with a live-stream playing in the room.
- Sunday 8:00 AM – 12:00 PM

8:30 AM - 5:30 PM

One-on-One Sessions – Fairmount Suite I

Meet for an individual consultation with Simons to discuss aspects of *STXBP1* Disorders that matter most to your family.

Also available:

- Sunday 8:00 AM – 12:00 PM

6:00 PM - 9:00 PM

Social Hour & Dinner Banquet with Families – Ben Franklin

- Social Hour / Poster Sessions
- Keynote Speaker & Dinner – Beverly Davidson, PhD; Children’s Hospital of Philadelphia
- Closing Remarks – Anne Johnson; STXBP1 Foundation

The SIFM was also made possible by:



Raymond G. Perelman Center for Cellular and Molecular Therapeutics

