

5th International RASopathies Symposium: When Development and Cancer Intersect

Renaissance Orlando at SeaWorld, Orlando, Florida ~ Friday, July 28 to Sunday, July 30, 2017

AGENDA

Chairs: Katherine A. Rauen, MD, PhD and Frank McCormick, PhD, FRS

Co-Organizers: RASopathiesNet - Lisa Schoyer, MFA, Lisa Schill, BS, Beth Stronach, PhD

DAY 1 – FRIDAY 7/28/17 – Atrium A & B

8:00-10:00 pm	Dessert and Scientific Poster Session (Scientists and advocacy/family groups) Goal: Encourage collaboration between researchers and families in a nonclinical setting
---------------	---

DAY 2 – SATURDAY 7/29/17 – Crystal E

7:00-8:00 am	Breakfast (Crystal D)
9:00 am-5:00 pm	Family Photo Shoot in and around meeting areas - Rick Guidotti
8:00-10:00 am	Session 1: What Defines a RASopathy? Moderator: Martin Zenker, MD Goals: Hear from patients and families about their experiences living with a RASopathy. Lay the scientific foundation for a comprehensive and accurate understanding of the current definition of a RASopathy.
8:00-8:30 am	Four Individuals/Caregivers: Perspectives from the Home Front
8:30-9:00 am	Frank McCormick, PhD, FRS: Molecular primer on the Ras signaling pathway
9:00-9:30 am	Katherine A. Rauen, MD, PhD: The RASopathies
9:30-10:00 am	<i>Discussion</i> (Clinical Diagnosis, Phenotype-Centered vs. Molecularly Defined)
10:00-10:10 am	<i>Break</i>
10:10 am -12:00 pm	Session 2: Syndromic and Sporadic Cancers of the Ras Pathway Moderator: Karen Gripp, MD Goal: Examine cancer types and mechanisms in individuals with RASopathies
10:10-10:50 am	KEYNOTE: Nancy Ratner, PhD: Preclinical studies to guide NF1 clinical trials
10:50-11:10 am	Corinne Linardic, MD: Pediatric sarcomas, rhabdomyosarcoma
11:10-11:30 am	Hélène Cavé, PharmD PhD: Myoproliferative neoplasms (JMML) in RASopathies
11:30-11:50 am	Brigitte Widemann, MD: Clinical trial updates, NF1 Plexiforms and MPNSTs
11:50 am – 12:00 pm	<i>Discussion</i>
12:00 – 1:00 pm	Lunch and Learn: Rick Guidotti: Positive Exposure (Crystal D)
1:00-4:30 pm	Session 3: Human Development: Effects on Organ Systems Moderator: Bruce Korf, MD, PhD Goal: Learn how RASopathy mutations affect developing tissues, organs, and body systems
1:00-1:40 pm	KEYNOTE: Marco Tartaglia, PhD: RASopathy genetics, new mutations, congenital defects
1:40-2:40 pm	NERVOUS SYSTEM
1:40-2:00 pm	Erika Yeh, PhD (Weiss lab): From skin cells to neurons
2:00-2:20 pm	Erik Ullian, PhD: How do astrocytes affect brain function in RASopathies?
2:20-2:40 pm	Giuseppe Zampino, MD: Penn MDBR 2015 Grantee - Pain in RASopathies: new investigative techniques and treatments
2:40-2:50 pm	<i>Discussion</i>
2:50-3:00 pm	<i>Break</i>
3:00-3:40 pm	CIRCULATORY SYSTEMS
3:00-3:20 pm	Bruce Gelb, MD: Human inducible pluripotent stem cells for the study of heart defects
3:20-3:40 pm	Max Itkin, MD: Imaging of the central lymphatic system in patients with Noonan syndrome using Dynamic Contrast Enhanced MR Lymphangiography
3:40-4:00 pm	GASTROINTESTINAL SYSTEM Cheng Sun, PhD (Kontaridis lab): Penn MDBR 2014 Grantee - Using human inducible pluripotent stem cells to delineate the cause of gastrointestinal abnormalities in RASopathy disorders
4:00-4:10 pm	<i>Discussion</i>
4:10-4:20 pm	<i>Break</i>

5th International RASopathies Symposium: When Development and Cancer Intersect Agenda, continued

4:20-5:50 pm	Session 4: Developmental Perspective: Modeling RASopathies in Animals and in Silico Moderator: Suma Shankar, MD, PhD Goal: Discuss nonhuman model experimental systems and how they are used to study RASopathies
4:20-4:40 pm	Stanislav Shvartsman, PhD: Quantitative biology of RASopathies
4:40-5:00 pm	Ethan Perlstein, PhD (Perlara): Rare disease drug discovery using whole animal disease models
5:00-5:20 pm	Edward Stites, MD, PhD: Computational analysis of pathological Ras mutants
5:20-5:40 pm	Annette Schenck, PhD: Habituation learning in <i>Drosophila</i> - a high-throughput platform to identify drugs that ameliorate cognitive and behavioral problems in Rasopathies
5:40-5:50 pm	<i>Discussion</i>
5:50-6:30 pm	<i>Break</i>
6:30-8:30 pm	Networking Dinner with Cash Bar

DAY 3 – SUNDAY 7/30/17 – Crystal E

7:00-8:00 am	Breakfast Meeting: How Do You Define a RASopathy? (Crystal D)
8:00-9:30 am	Session 5: Ras Pathway Mechanics Moderator: Martin McMahon, PhD Goal: To discuss molecular mechanisms of Ras pathway signaling: How do crystal structures and dynamic imaging shed new light?
8:00-8:20 am	William Huang, PhD: Membrane signaling dynamics
8:20-8:40 am	John Albeck, PhD: Quantifying the cellular effects of Ras pathway mutations with live-cell imaging
8:40-9:00 am	Deborah Morrison, PhD: Divide and Conquer: Targeting Raf Regulatory Interactions
9:00-9:20 am	Marc Therrien, PhD: Allosteric control of RAF activation by dimerization
9:20-9:30 am	<i>Discussion / Break</i>
9:30-11:00 am	Session 6: Potential Therapeutics Moderator: David Stevenson, MD Goal: Discuss promises and challenges of Ras pathway therapeutic drug development
9:30-9:50 am	Brage Andresen, PhD, FRCPATH: Splice switching oligonucleotides (SSOs) for HRAS exon 2 skipping
9:50-10:10 am	Steven Fruchtman, MD (Onconova Therapeutics): Rigosertib for JMML
10:10-10:30 am	Christopher Gibson, MD, PhD (Recursion Pharmaceuticals): Image-based high throughput screens for rare disease therapeutics
10:30-10:50 am	Philip Stork, MD: Penn MDBR 2016 Grantee - What can Ras-dependent cancers teach us about Rasopathies?
10:50-11:00 am	<i>Discussion / Break</i>
11:00-12:30 am	Session 7: Next Generation: Junior Investigator Poster Session Abstracts and Closing Keynote Moderator: Bronwyn Kerr, MBBS Goal: Attract and highlight research on RASopathies from junior investigators
11:00-11:30 am	Junior Investigator Poster Finalists: Three 10-minute presentations, winner announced
11:30-12:20 pm	KEYNOTE: Frank McCormick, PhD, FRS: Targeting Ras and NF1-related malignancies
12:20-12:30 pm	<i>Discussion and Next Steps</i> Frank McCormick, PhD, FRS, Katherine A. Rauen, MD, PhD, Lisa Schoyer, MFA
12:30-1:30 pm	Breakout Sessions (Coral A, B, C, Labrid B) For RASopathy Family Groups: CFC: Katherine A. Rauen, MD, PhD CS: Karen Gripp, MD, David Stevenson, MD, and Bronwyn Kerr, MBBS NS: Bruce Gelb, MD, and Amy Roberts, MD NIH Q&A: William C. Timmer, PhD, NCI Program Director
12:30-4:30 pm	RASopathy Advocacy Organizations' Meeting on Approaching Data Collecting and Sharing
12:30-6:00 pm	NF Network's Post-Symposium Family Meeting
2:30-6:00 pm	Post-Symposium Events for CFC and NS families