

2015 RASopathies Symposium Agenda

July 17-19, 2015
 Doubletree by Hilton at SeaTac

Summary of free sessions for families can be found at the end of this document.

DAY 1 – Friday, July 17, 2015 -- Northwest 1 and 2

8:00-10:00 pm	Dessert and Scientific Poster Session (Scientists and advocacy/family groups) Goal: Encourage collaboration between researchers and families in a non-clinical setting
8:00-8:15 pm	Welcoming Comments: David Stevenson, Brigitte Widemann, Lisa Schoyer

DAY 2 – Saturday, July 18, 2015 -- Northwest 2 and 3

7:00-8:00 am	Meet the Expert Breakfast (Grand 2)	
8:00-8:15 am	Introduction: David Stevenson, Brigitte Widemann	
8:15-9:00 am	Keynote Presentation: “Ras-signaling and stem-ness” Frank McCormick (UCSF Helen Diller Family Comprehensive Cancer Center)	
9:00am-5:00pm	Photo Shoot in and around CFC International and CSFN meeting areas Rick Guidotti	
9:00-10:00 am	RASopathy Phenotypes (Shared and Discordant) Moderators: Bronwyn Kerr, Chiara Leoni Goal: Describe phenotypic overlap, with focus on translation of therapies and potential measurable endpoints for clinical trials	
9:00-9:15 am	Cancer and the RASopathies	Bruce Korf (University of Alabama, Birmingham)
9:15-9:30 am	Mechanisms underlying cognitive deficits in the RASopathies: from mice to trials	Ype Elgersma (Erasmus Medical Center, The Netherlands)
9:30-9:45 am	Cardiac Manifestations in RASopathy Syndromes	Kathryn Chatfield (University of Colorado)
9:45-10:00 am	Questions and discussion	
10:00-10:30 am	Advocates’ Panel Moderator: Lisa Schoyer Representative from each syndrome group will address the pain they experience, to help identify endpoints for clinical trials: CFC: Judy Doyle; CS: Angel Thomas; NF1: Rosemary Anderson; NS: Michelle Ellis; NSML/LS: Tammy Bowers	
10:30-10:45 am	Break	

10:45-12:30 pm	Preclinical Studies Moderators: Florent Elefteriou, Anne Goriely Goal: Discuss models of RASopathies and approaches to preclinical drug discovery and testing	
10:45-11:05 am	Treating RASopathies	Katherine A. Rauen (University of California at Davis)
11:05-11:25 am	A Chemical Screen for Hits for Noonan Syndrome Using a Fruit Fly Model	Bruce Gelb (Mt. Sinai School of Medicine)
11:25-11:45 am	Developing therapies for rare tumors: using mouse models of malignant peripheral nerve sheath tumors to complement rare human samples in drug screens. Screening for MPNST therapy	Karlyne Reilly (NIH National Cancer Institute)
11:45-12:05 pm	Splicing of HRAS exon 2 is vulnerable – Importance for Costello Syndrome phenotype and potential treatment by splice shifting oligonucleotides	Brage Andresen (University of S. Denmark)
12:05-12:20 pm	Treatment of stature using CNP	Florent Elefteriou (Vanderbilt University)
12:20-12:30 pm	Questions and Discussion	
12:30-1:30 pm	Lunch Break -- Grand 2	
1:30-3:10 pm	Clinical Trials in RASopathies: Design, Endpoints, and Results Moderators: Scott Plotkin, Emma Burkitt-Wright Goal: Discuss ongoing and planned trials: Success, pitfalls, challenges	
1:30-1:50 pm	Therapeutics for NF1: “The promise of MEK”	Brigitte Widemann (NIH National Cancer Institute)
1:50-2:10 pm	Pilot Trial of Effect of RAS/MAPK inhibitors on Neurocognitive Function in NF1	Karin Walsh (Children’s National Medical Center)
2:10-2:30 pm	Therapeutics for Rare and Neglected Diseases (TRND) and Noonan Syndrome with Multiple Lentigines (NSML)	Amy Roberts (Children’s Hospital Boston)
2:30-2:50 pm	Individualized endpoint analysis for use in clinical trial of neurofibromatosis	Scott Plotkin (Massachusetts General Hospital)
2:50-3:10 pm	Questions and Discussion	
3:10-3:25 pm	Break	
3:25-5:30 pm	Collaborations, Industry, and Funding Moderators: Annette Bakker, Lisa Schill Goal: Discuss mechanisms for collaborations translating into clinical trials	
3:25-3:40 pm	Funding Patient-centered Outcomes Research for Rare Disorders	Suzanne Schrandt (PCORI)

3:40-4:00 pm	Agents targeting RAS/MAPK in clinical development: Current status	Michael Fisher (Children's Hospital Philadelphia)
4:00-4:15 pm	NCI Rare Tumors Initiative	Abby Sandler (NIH National Cancer Institute)
4:15-4:35 pm	Children's Tumor Foundation Business model	Annette Bakker (Children's Tumor Foundation)
4:35-5:30 pm	Panel discussion with industry representatives	Alexion - Andre Marozsan (Interim Director, Preclinical Pharmacology); Cellerant - Ram Mandalam (President & CEO); Plexxikon - Marguerite Hutchinson (Director, Business Development); Synlogic - Alison Silva (EVP, COO); UC Davis - Katherine A. Rauen (MD, PhD)
5:30pm Plus	Free Time - Networking	

Day 3 – Sunday, July 19, 2015

7:00-8:00 am	Breakfast -- Grand 2	
8:00-9:00 am	Abstracts by Young Investigator Finalists -- Northwest 2 and 3 Moderators: Karen Gripp, Amy Roberts Goal: Provide a forum for trainees to present their research and encourage participation	
8:15-8:30 am	<i>BRAF</i> knock-in mice provide a pathogenetic mechanism of developmental defects and a therapeutic approach in RASopathies	Shin-ichi Inoue
8:30-8:45 am	Social functioning in children with neurofibromatosis type 1 (NF1) and Noonan syndrome (NS)	Rene Pierpont
8:45-9:00 am	Taking a closer look into Costello Syndrome	Katherine Robbins
9:00-10:00 am	Positive Exposure – Seeing Beyond Diagnosis -- Northwest 1 Rick Guidotti	
9:00-11:00 am	RASopathy Cousins -- Northwest 2 and 3 Moderator: Dawn Siegel Goals: To discuss lessons learned from other conditions that intersect the Ras pathway	
9:00-9:30 am	PIK3CA -Related Overgrowth Spectrum (PROS): Clinical & Molecular Characterization, Evaluation and Potential Therapeutic Interventions	Kim Keppler-Noreuil (NIH National Human Genome Research Institute)
9:30-10:00 am	Clinical and Molecular Findings of RASA1-Related Disorders	Pinar Bayrak-Toydemir (University of Utah)

10:00-10:30 am	TSC	David Franz (University of Cincinnati)
10:30-11:00 am	PTEN Project Summary	Antonio Hardan (Stanford University)
11:00-11:30 am	Final Comments and Discussion David Stevenson, Brigitte Widemann, Lisa Schoyer	
11:30-12:30 pm	Breakout Sessions with RASopathies Groups: CFC: Katherine A. Rauen, MD, PhD -- Evergreen 2 CS: Karen Gripp, MD, and David Stevenson, MD -- Evergreen 1 NF1: Bruce Korf, MD, PhD -- Evergreen 3 NS: Bruce Gelb, MD, and Amy Roberts, MD -- Evergreen 4	
12:30-2:30 pm	Research Networking Lunch for academic and industry professionals	
12:30-3:00 pm	NF Network's Post-Symposium Meeting-- Evergreen 3 – Lunch for NF1 families included Bruce Korf, MD, PhD and David Stevenson, MD	
2:00-4:00 pm	Post-Symposium Noonan Syndrome Meeting Rene Pierpont, PhD and Patroula Smpokou, MD -- Northwest 1	

Summary of Free Sessions for Families

DAY 1 – Friday, July 17, 2015

8:00-10:00 pm	Dessert and Scientific Poster Session – Northwest 1 and 2 For scientists and advocacy/family groups Goal: Encourage collaboration between researchers and families in a non-clinical setting
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Day 2 – Saturday, July 18, 2015

9:00am-5:00pm	Photo Shoot -- around Evergreen 1, 2, 3 and 4 Rick Guidotti
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Day 3 – Sunday, July 19, 2015

9:00-10:00 am	2015 Symposium RASopathies Photography Rick Guidotti
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1:30-12:30 pm	Breakout Sessions with RASopathies Groups: CFC: Katherine A. Rauen, MD, PhD – Evergreen 2 CS: Karen Gripp, MD, and David Stevenson, MD Evergreen 1 NF1: Bruce Korf, MD, PhD – Evergreen 3 NS: Bruce Gelb, MD, and Amy Roberts, MD – Evergreen 4
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12:30-3:00 pm	NF Network's Post-Symposium Meeting – Evergreen 3 – Lunch for NF1 families included Bruce Korf, MD, PhD and David Stevenson, MD
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2:00-4:00 pm	Noonan Syndrome Post-Symposium Meeting – Northwest 1 Rene Pierpont, PhD and Patroula Smpoku, MD
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