

2015 RASopathies Symposium Agenda

- DRAFT -

DAY 1 – Friday, July 17, 2015

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, 2014

7:00-8:00 am	Meet the Expert Breakfast	
8:00-8:15 am	Introduction: David Stevenson, Brigitte Widemann	
8:15-9:00 am	Keynote Presentation: “Ras-driven Cancers” – Frank McCormick (Frederick National Laboratory for Cancer Research)	
9:00-10:15 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	
9:00-9:20 am	Overview of the RASopathies	David Stevenson (University of Utah)
9:20-9:35 am	Cancer	Bruce Korf (University of Alabama, Birmingham)
9:35-9:50 am	Cognition/Learning	TBA
9:50-10:05 am	Cardiac manifestations	Kathryn Chatfield (University of Colorado)
10:05-10:15 am	Questions and discussion	
10:15-10:30 am	Break	
10:30-10:45 am	Advocates’ Panel Moderators: Lisa Schoyer, Tammy Bowers. Representative from each syndrome group will present what the respective groups consider the top 5 manifestations they wish would be targeted for clinical trials.	
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	MEK inhibitors for strength	Katherine A. Rauen (UC Davis)
11:05-11:25 am	Drug Target screening – Drosophila	Bruce Gelb (Mt. Sinai School of Medicine)
11:25-11:45 am	NFI preclinical models and trials	Karlyne Reilly (NIH National Cancer Institute)
11:45-12:05 pm	Alternative Splicing Therapeutics – HRAS	Brage Andresen (University of S. Denmark)
12:05-12:30 pm	(Discussion, or selected from abstracts)	
12:30-1:30 pm	Lunch Break	
1:30-3:40 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	MEK inhibition for cardiomyopathy in NS	TBA
1:50-2:10 pm	Phase I trial of AZD6244 in NFI tumors	Brigitte Widemann (NIH National Cancer Institute)
2:10-2:30 pm	Pilot trial of effect of RAS/MAPK inhibitors on neurocognitive function in NFI	Karin Walsh (Children’s National Medical Center)

2:30-2:50 pm	TRND study	Amy Roberts (Children's Hospital Boston)
2:50-3:10 pm	Trial Design using individualized endpoints	Scott Plotkin (Massachusetts General Hospital)
3:10-3:40 pm	Break	
3:40-5:30 pm	Collaborations, Industry, and Funding Moderators: Annette Bakker, Lisa Schill Goal: Discuss mechanisms for collaborations translating into clinical trials	
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 Disease Initiative	Abby Sandler (NIH National Cancer Institute)
4:15-4:35 pm	Performing clinical trials with small patient numbers	Shivaani Kummar (NIH National Cancer Institute)
4:35-4:55 pm	Business Model	Annette Bakker (Children's Tumor Foundation)
4:55-5:45 pm	Panel discussion with industry representatives	[potential participants are being considered at this time]
6:30-8:00 pm	Dinners with RASopathy Support Groups Participants will be encouraged to partake in the concurrent family forum dinners	

Day 3 – Sunday, July 19, 2014

7:00-8:00 am	Breakfast	
8:00-9:00 am	Abstracts by Young Investigators Moderators: Karen Gripp, Amy Roberts Goal: Provide a forum for trainees to present their research and encourage participation	
8:15-9:00 am	15-minute presentations from top Young Investigator abstracts	
9:00-11:00 am	RASopathy Cousins Moderators: Dawn Siegel, TBA Goals: To discuss lessons learned from other conditions that intersect the Ras pathway	
9:00-9:30 am	PIK3CA conditions	Kim Keppler-Noreuil (NIH National Human Genome Research Institute)
9:30-10:00 am	RASA1	Pinar Bayrak-Toydemir (University of Utah)
10:00-10:30 am	TSC	David Franz (University of Cincinnati)
10:30-11:00 am	PTEN	Charis Eng (Cleveland Clinic)
11:00-11:30 am	Final Comments and Discussion David Stevenson, Brigitte Widemann, Lisa Schoyer	
11:30-12:30 pm	Breakout Sessions with Advocacy Groups	
12:30-2:30 pm	Networking and Satellite Opportunities	
12:30-5:00 pm	NFI and Noonan Syndrome family post-conference meetings with clinicians and researchers	