

RASopathies Symposium Draft Agenda

<i>tentative schedule</i>	<i>description</i>	<i>speaker/moderator</i>
F 8-10pm	Dessert Reception and Poster Session	
8:00-8:15pm	Welcome Remarks	Karen Gripp , MD, Nemours/AI duPont Hospital for Children Nancy Ratner , PhD, Cincinnati Children's Hospital Medical Center Lisa Schoyer , Lisa Schill, Elisabeth Parker, and Beth Stronach, RASopathies Network
SA 7:00-8:00am	Breakfast	
SA 8:00-8:20am	Introductory Remarks: The RASopathies Umbrella	Katherine A. Rauen , MD, PhD, UC Davis, CA
SA 8:20-10:05am	Session 1: Novel Mutations/Genes in the Field	Moderator: Marco Tartaglia , PhD, Ospedale Pediatrico Bambino Gesù, Rome, Italy
8:20-8:35	LZTR1 - Phenotypic overlap with RASopathies	Martin Zenker , MD, University Hospital of Magdeburg, Germany
8:35-8:50	Mutations in LZTR1 drive human disease by dysregulating RAS ubiquitination and	Anna Sablina , PhD VIB-KU Leuven Center for Cancer Biology, Leuven, Belgium
8:50-9:05	The SHOC2-MRAS-PP1 complex: Molecular Details and Pathway Function	Pablo Rodriguez-Viciana , PhD, University College, London, UK
9:05-9:20	PPP1CB - Phenotypic Overlap with RASopathies	Karen Gripp , MD, Nemours/AI duPont Hospital for Children
9:20-9:35	Biology of SYNGAP1-related Neurodevelopmental Disorders: Should They Be Co	Gavin Rumbaugh , PhD, Scripps Research Institute, FL
9:35-9:50	SYNGAP1 - Phenotypic Overlap with RASopathies	Jimmy Holder , MD PhD, Baylor College of Medicine, TX
9:50-10:05	<i>Discussion over Coffee</i>	
SA 10:05am-11:25am	Session 2: New Functions of RASopathy Genes	Moderator: Emma Burkitt-Wright , MD, PhD, NHS Foundation Trust, Manchester, UK
10:05-10:20	Metabolic Effects in Mouse Model of Costello Syndrome	Shin-ichi Inoue , PhD, Tohoku U School of Medicine, Japan
10:20-10:45	Targeting SHP2 for Neurofibromatosis and Other Malignancies	Ben Neel , MD PhD, NYU Langone Med Ctr, NY
10:45-11:10	Structural and functional analysis of neurofibromin and SPRED1	Frank McCormick , PhD FRS DSc, UCSF, CA
11:10-11:25	<i>Discussion</i>	
SA 11:25am-12:15pm	KEYNOTE: The inhibitor landscape and Ras signaling feedback loops	Neal Rosen , MD PhD, MSKCC, NYC
SA 12:20-1:20pm	LUNCH - Presentation: Steph Nimmo, Author "Was This in the Plan?"	
SA 1:30-3:05pm	Session 3: Therapeutic Inhibitors	Moderator: William Timmer , NCI (CTEP)
1:30-1:50	Cancer Driven by HRAS: Tipifarnib Clinical Trial Results	Alan L. Ho , MD PhD, MSKCC, NYC
1:50-2:10	Functional Endpoints on the SPRINT Study	Andrea Gross , MD, NCI Center for Cancer Research (CCR)
2:10-2:25	Low-Dose Dasatinib for Cardiomyopathy in Noonan Syndrome	Anton Bennett , PhD, Yale, CT
2:25-2:45	Drugging RAS from Multiple Angles	Darryl McConnell , PhD, Boehringer-Ingelheim, Austria
2:45-3:05	<i>Discussion over Coffee</i>	
SA 3:10-5:15pm	Session 4: Potential Endpoints for Clinical Trials in Rasopathies	Moderator: Nancy Ratner , PhD, Cincinnati Children's Hospital
	The Brain in RASopathies	
3:10-3:25	Temporal dynamics of contextual memory linking in PTPN11D61G mutant mice	Alcino Silva , PhD, UCLA, CA
3:25-3:40	Antioxidant Testing in Preclinical Models and RASopathy Patients	Carlos Prada , MD, Cincinnati Childrens Hospital
3:40-3:55	Bridging the gaps in our knowledge: brain development of children with	Tamar Green , MD, Stanford University, CA
3:55-4:10	Neuroimaging of RASopathies in the newborn and young child	Susan Blaser , MD FRCP, Hospital for Sick Children, Toronto, Canada
	Cardiac Function in RASopathies	
4:10-4:25	iPSC-derived cardiomyocytes reveal aberrant ERK5 and MEK1/2 signaling	Maria Kontaridis , PhD, Masonic Medical Research Lab, Utica, NY
4:25-4:40	Unraveling the Cause of Developmental Disease Using Zebrafish: MAP4K4 Trun	Rebecca Burdine , PhD, Princeton, NJ
4:40-5:10	<i>Discussion</i>	
	Free time	
SA 6:00-8:00pm	NETWORKING DINNER or CSFN Family Tailgate Party	
SU 7:00-8:00am	Breakfast	
SU 8:00-8:50am	Session 5: Prenatal Findings, Manifestations, Diagnosis and Management	Moderator: Pilar Magoulas , MS CGC, Baylor College of Medicine, TX
8:00-8:40	Panelists:	Sandra Darilek MS CGC, Baylor; Angie Jelin MD- MFM, Johns Hopkins
8:40-8:50	<i>Discussion</i>	
SU 8:50-10:40am	Session 6: Clinical Trial Endpoints Roundtable	Moderator: Richard Klein , former Director of FDA's Patient Liaison Program, WDC Area
8:50-9:05	PPMD: Forefront of PRO	Annie Kennedy , Parent Project Muscular Dystrophy, MD
9:05-9:20	Benefits, challenges, and strategies for incorporating patient-reported outcome	Pam Wolters , PhD, NCI, NIH
9:20-9:35	A prospective study of the impact of MEK1/2 inhibition on neurocognitive funct	Karin Walsh , PsyD, Children's Research Inst., WDC [NF1 REINS Consortium]
9:40-10:10	Advocacy Organizations Panel	Moderator: Michelle Ellis , Noonan UK; Panelists: Tuesdi Dyer , CFCI; Gregg Erikson , NF Network; Alwyn Dias , CTF; Amanda Brown , NSF; Angel Thomas , CSFN
10:10-10:40	<i>Discussion</i>	
SU 10:45am-12:15pm	Session 7: Junior Investigators, MDBR Grantees, Closing Comments	Moderator: Amy Roberts , MD, Harvard Medical School, Boston Children's Hospital
10:45-11:00	Jr Investigator Finalist 1: Modeling LZTR1 Loss-of-Function in Vivo: A Novel Noo	Pau Castel , PhD, University of California San Francisco, CA
11:00-11:15	Jr Investigator Finalist 2: Hematopoietic Defects in a Zebrafish Knock-in Model c	Maja Solman , PhD, Hubrecht Institute, The Netherlands
11:15-11:30	Jr Investigator Finalist 3: SHP2/PTPN11 Interactions with Protein Zero Related (I	Jae-Sung Yi , PhD, Yale, New Haven, CT
11:30-11:45	Million Dollar Bike Ride 2018 Grantee: Using a clinically approved antiemetic, IV	Kartik Venkatachalam , PhD, UT Houston, TX
11:45-12:00	Million Dollar Bike Ride 2019 Grantee: Advancing a Novel Therapeutic Lead for	Bruce Gelb , MD, Mt. Sinai School of Medicine, NY
12:00-12:15	<i>Discussion and Closing Remarks</i>	
SU 12:30-1:30pm	Breakout Sessions	
12:30-1:30pm	Breakout for CFC Families	Pilar Magoulas , MS CGC, Dave Stevenson , MD
12:30-1:30pm	Breakout for CS Families - Hosted by CSFN	Karen Gripp , MD, Emma Burkitt-Wright , MD, PhD
12:30-1:30pm	Breakout for NS, NSML Families	Bruce Gelb , MD, Amy Roberts , MD
12:30-3:30 pm	Breakout for NF1 Families - Hosted by NF Network	Bruce Korf MD, PhD, Karin Walsh PsyD, Pam Wolters PhD
2:30-4:00pm	RASopathies Network Open Meeting	Board members from RASNet and other family support organizations