



9th International RASopathies Symposium

July 25-27, 2025, Orlando, FL

Draft Agenda - 03/13/25

Description	Speaker	Duration	Schedule
FRIDAY Opening Night Dessert Reception, Posters, and Keynote 1			
Welcome & Awards	Pau Castel, Tamar Green, Lisa Schoyer, Beth Stronach	15m	7:00-7:15pm
Poster Session		1h	7:15-8:15pm
Keynote 1: Progress on NF1- Insights for RASopathies	Jaishri Blakeley, MD / Johns Hopkins	45m	8:15-9:00pm

SATURDAY Breakfast		1h	7:30am
I-Systems 1: Feeding and Gastrointestinal Function			
<i>Moderator: Katherine Rauen, MD, PhD / UC Davis</i>			
Family perspective	Dagmar Tiemens / Radboud UMC	10m	8:30-8:40am
Pediatric gastrointestinal neuromodulation	Khalil El-Chammas, MD / Cincinnati Children's	20m	8:40-9:00am
Intestinal organoid system to study rare pediatric intestinal disorders	Abdul Ekadri, MD / Medical College Wisconsin	20m	9:00-9:20am
Botox improves chronic nausea and vomiting in children with disorder of gut-brain interaction	John Fortunato, MD / Lurie Children's	20m	9:20-9:40am
Metabolic profiling in NF1	Miriam Bornhorst, MD / Lurie Children's	20m	9:40-10:00am
Feeding avoidance in RASopathies	Keir Shiels, MD & Angharad Roberts MD, PhD / GOSH, London	20m	10:00-10:20am
ESI Selected Abstract	TBD	10m	10:20-10:30am
Break		20m	10:30-10:50am
II-Systems 2: Neurodevelopment, Brain, and Behavior			
<i>Moderator: Tamar Green, MD / Stanford</i>			
Family perspective	TBD	10m	10:50-11:00am
Mechanism of learning disability associated with RASopathies	Yong-Seok Lee, PhD / Seoul National University College of Medicine	20m	11:00-11:20am
Perspectives of caregivers & young adults with RASopathies on neurodevelopmental and mental health treatments	Rene Pierpont, PhD / University of Minnesota Medical School	20m	11:20-11:40am
SOAR: Social Outcomes in Adults with RASopathies	Jonathan Payne, DPsych / Murdoch Children's Research Institute	20m	11:40am-12:00pm
ESI Selected Abstract	TBD	10m	12:00-12:10pm
Lunch and Poster Session Redux		1h30m	12:15pm
III-Systems 3: Cardiovascular and Lymphatic Function			
<i>Moderator: **Bruce D. Gelb, MD / Mount Sinai</i>			
Family perspective	Tammy Bowers / Noonan Syndrome Foundation	10m	1:45-1:55pm
Novel mechanisms of PTPN11 signaling in Noonan syndrome	Anton Bennett, PhD / Yale School of Medicine	20m	1:55-2:15pm
HCM organoid models for high-throughput screens	**Fabrice Jaffre, PhD / Monash University	20m	2:15-2:35pm
Outcomes of MEKi treatment for cardiac and lymphatic complications	Katherine Chatfield, MD, PhD / Children's Hospital Colorado	20m	2:35-2:55pm
Precision medicine for RASopathy vascular anomalies	Sarah E. Sheppard , MD, PhD, MSTR / NICHD	20m	2:55-3:15pm
ESI Selected Abstract	TBD	10m	3:15-3:25pm
Break		30m	3:25-3:55pm



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IV-RASopathies Genetics			
<i>Moderator: Karen Gripp, MD / Nemours</i>			
Family perspective	TBD	10m	3:55-4:05pm
Paternal age effects and selfish spermatogonial selection	Anne Goriely, PhD / Oxford	20m	4:05-4:25pm
Variant-phenotype correlations in RASopathies	Alessandro De Luca, PhD / IRCSS-Fondazione Casa Sollievo della Sofferenza	20m	4:25-4:45pm
Evolving genetic landscape of RASopathies	Marco Tartaglia, PhD / Ospedale Pediatrico Bambino Gesù	20m	4:45-5:05pm
ESI Selected Abstract	TBD	10m	5:05-5:15pm
<i>Free Time</i>		1h15	5:15-6:30pm
Networking Dinner		2h	6:30-8:30pm

SUNDAY Breakfast		1h	7:00am
V-Predisposition to Malignancy			
<i>Moderator: Marielle Yohe, MD, PhD / NCI</i>			
Family perspective	TBD	10m	8:00-8:10am
T cell involvement in NF1 tumorigenesis	Nancy Ratner, PhD / Cincinnati Children's	20m	8:10-8:30am
Natural History of Non-NF1 RASopathies	Gina Ney , MD, PhD / CGB DCEG, NCI	20m	8:30-8:50am
Cancer in Costello syndrome	Doug Stewart, MD / CGB DCEG, NCI	20m	8:50-9:10am
Mosaicism to Cancer Continuum	Christian Kratz, MD / Hannover Medical School	20m	9:10-9:30am
Melanocytic Nevi and Melanoma	Maija Kiuru, MD, PhD / UC Davis	20m	9:30-9:50am
ESI Selected Abstract	TBD	10m	9:50-10:00am
<i>Break</i>		15m	10:00-10:15am
VI-Therapeutic Pipeline			
<i>Moderator: Pau Castel, PhD / NYU Langone</i>			
Keynote 2:	Frank McCormick, PhD, FRS / UCSF	45m	10:15-11:00am
Outlook for MEKi trial for Ras-associated HCM	**Gregor Andelfinger , MD, PhD / CHU Sainte-Justine	20m	11:00-11:20am
Panel: Industry POV & Pipelines	TBD	55m	11:20am-12:15pm
Discussion	McCormick, Blakely, Andelfinger, Industry Reps	30m	12:15-12:45pm
Closing Remarks	Pau Castel, Tamar Green, Beth Stronach, Lisa Schoyer	5m	12:45pm
Symposium Summary Sessions			1pm-
for CFC Families - Hosted by CFC International	Rene Pierpont		
for CS Families - Hosted by Costello Syndrome Family Network (CSFN)	David Stevenson, Karen Gripp		
for NS, NSML, NSLAH Families - Hosted by Noonan Syndrome Foundation (NSF) & Noonan Syndrome Association (NSA)	Bruce Gelb, Gregor Andelfinger		
for NF1 Families - Hosted by NF Network	Jaishri Blakely		

** = Penn Orphan Disease Center's Million Dollar Bike Ride RASopathies Research Grant Awardee;

bold = Early stage investigator