

9th International RASopathies Symposium July 25-27, 2025, Orlando, FL

Draft Agenda - 03/13/25

Description	Speaker	Duration	Schedule
FRIDAY Opening Night Dessert Recep	otion, 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

I-Systems 1: Feeding and Gastrointestinal Function Moderator: Katherine Rauen, MD, PhD / UC Davis Family perspective Dagmar Tiemens / Radboud UMC 10m 8:30-8:40am Perspective John Fortunato, MD / Cincinnati Children's 20m 8:40-9:00am Intestinal organoid system to study rare pediatric intestinal disorders and stock improves chronic nausea and vomiting in children with disorder of gut-brain interaction Metabolic profiling in NF1 Miriam Bornhorst, MD / Lurie Children's 20m 9:20-9:40am interaction Metabolic profiling in NF1 Miriam Bornhorst, MD / Lurie Children's 20m 9:40-10:00am Feeding avoidance in RASopathies (Fishiels, MD & Angharad Roberts MD, PhD / 20m 10:00-10:20am (GOSH, London 20m) ESI Selected Abstract TBD 10m 10:20-10:30am Break 20m 10:30-10:50am III-Systems 2: Neurodevelopment, Brain, and Behavior Moderator: Tamar Green, MD / Stanford Family perspective TBD 10m 10:50-11:00am Mechanism of learning disability associated with RASopathies on neurodevelopmental and mental health treatments SOAR: Social Outcomes in Adults with Rasopathies Research Institute 10m 12:00-12:10pm Lunch and Poster Session Redux III-Systems 3: Cardiovascular and Lymphatic Function Moderator: **FBruce D. Gelb, MD / Mount Sinai Family perspective Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm Novel mechanisms of PTPN11 signaling in Anton Bennett, PhD / Yale School of Medicine 20m 1:55-2:15pm Novel mechanisms of PTPN11 signaling in Anton Bennett, PhD / Yale School of Medicine 20m 1:55-2:55pm Colcorado Sarah E. Sheppard, MD, PhD / Children's Hospital 20m 2:35-2:55pm Colcorado Sarah E. Sheppard, MD, PhD / Children's Hospital 20m 2:55-3:15pm Free Sielected Abstract TBD 10m 3:15-3:25pm Sielected Abstract TBD 10m 3:15-3:25pm Sielected Abstract TBD 10m 3:15-3:25pm	OATUDDAY Duranteen		1h	7:20om
Family perspective Dagmar Tiemens / Radboud UMC 20m 8:30-8:40am Pediatric gastrointestinal neuromodulation Intestinal organoid system to study rare pediatric intestinal disorders Abdul Ekadri, MD / Medical College Wisconsin 20m 9:20-9:40am Intestinal disorders Abdul Ekadri, MD / Medical College Wisconsin 20m 9:20-9:40am Intestinal disorders Botox improves chronic nausea and vomiting in children with disorder of gut-brain interaction Interaction Metabolic profiling in NP1 Miriam Bornhorst, MD / Lurie Children's 20m 9:40-10:00am Esil Selected Abstract TBD 20m 10:00-10:20am 20m 20m 10:00-10:20am 20m 20m 10:00-10:20am 20m 20m 10:00-10:20am 20m 20m 20m 20:00-10:20am 20m 20:00-10:20am 20m 20:00-10:20am 20m 20:00-10:20am 20m 20m 20:00-10:20am 20m 20m 20:00-10:20am 20m 20m 20:00-10:20am 20:00-	SATURDAY Breakfast		1h	7:30am
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Intestinal organoid system to study rare pediatric intestinal disorders Botox improves chronic nausea and vomiting in children with disorder of gut-brain interaction Metabolic profiling in NF1 Miriam Bornhorst, MD / Lurie Children's Keir Shiels, MD & Angharad Roberts MD, PhD/ GOSH, London ESI Selected Abstract TBD 10m 10:20-10:30am Break II-Systems 2: Neurodevelopment, Brain, and Behavior Moderator: Tamar Green, MD / Stanford Family perspective TBD 10m 10:50-11:00am 11:50-11:00am 12m 130-10:50am 11-00-11:20am 12m 130-11:20am 130-11:20am	Family perspective	Dagmar Tiemens / Radboud UMC	10m	8:30-8:40am
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Break 20m 10:30-10:50am	Feeding avoidance in RASopathies		20m	10:00-10:20am
III-Systems 2: Neurodevelopment, Brain, and Behavior Moderator: Tamar Green, MD / Stanford	ESI Selected Abstract	TBD	10m	10:20-10:30am
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### Moderator: Tamar Green, MD / Stanford Family perspective TBD 10m 10:50-11:00am	II-Systems 2: Neurodevelopment, Bra	ain, and Behavior		
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College of Medicine Perspectives of caregivers & young adults with RASopathies on neurodevelopmental and mental health treatments SOAR: Social Outcomes in Adults with RASopathies on neurodevelopmental and mental health treatments SOAR: Social Outcomes in Adults with RASopathies Research Institute ESI Selected Abstract TBD Jonathan Payne, DPsych / Murdoch Children's 20m 11:40am-12:00pm ESI Selected Abstract TBD 10m 12:00-12:10pm Lunch and Poster Session Redux III-Systems 3: Cardiovascular and Lymphatic Function Moderator: **Bruce D. Gelb, MD / Mount Sinai Family perspective Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm Novel mechanisms of PTPN11 signaling in Noonan syndrome HCM organoid models for high-throughput screens Outcomes of MEKi treatment for cardiac and lymphatic Colorado Precision medicine for RASopathy vascular anomalies ESI Selected Abstract TBD 10m 3:15-3:25pm	Family perspective	TBD	10m	10:50-11:00am
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RASopathies Research Institute 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 Moderator: **Bruce D. Gelb, MD / Mount Sinai Family perspective Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm Novel mechanisms of PTPN11 signaling in Noonan syndrome HCM organoid models for high-throughput screens Outcomes of MEKi treatment for cardiac and lymphatic complications Colorado Precision medicine for RASopathy vascular anomalies ESI Selected Abstract TBD 10m 3:15-3:25pm	Perspectives of caregivers & young adults with RASopathies on neurodevelopmental and mental health treatments		20m	11:20-11:40am
Lunch and Poster Session Redux III-Systems 3: Cardiovascular and Lymphatic Function Moderator: **Bruce D. Gelb, MD / Mount Sinai Family perspective Tammy Bowers / Noonan Syndrome Foundation Novel mechanisms of PTPN11 signaling in Noonan syndrome Noonan syndrome HCM organoid models for high-throughput screens Outcomes of MEKi treatment for cardiac and lymphatic complications Precision medicine for RASopathy vascular anomalies ESI Selected Abstract TBD Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm 1:45-1:55pm 10m 1:45-1:55pm 1:45-1:45pm 1:45-1:55pm 1:45-1:45pm 1:45-1:55pm 1:45-1:45pm 1:45-1:55pm 1:45-1:45pm 1:45-1:55pm 1:45-1:55pm 1:45-1:45pm 1:45-1:	SOAR: Social Outcomes in Adults with RASopathies		20m	
III-Systems 3: Cardiovascular and Lymphatic Function Moderator: **Bruce D. Gelb, MD / Mount Sinai Family perspective Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm Novel mechanisms of PTPN11 signaling in Noonan Syndrome Foundation 20m 1:55-2:15pm Noonan syndrome HCM organoid models for high-throughput screens Outcomes of MEKi treatment for cardiac and lymphatic complications Precision medicine for RASopathy vascular anomalies ESI Selected Abstract TBD Tammy Bowers / Noonan Syndrome Foundation 10m 1:45-1:55pm 1:45-1:45pm	ESI Selected Abstract	TBD	10m	12:00-12:10pm
Moderator: **Bruce D. Gelb, MD / Mount SinaiFamily perspectiveTammy Bowers / Noonan Syndrome Foundation10m1:45-1:55pmNovel mechanisms of PTPN11 signaling in Noonan syndromeAnton Bennett, PhD / Yale School of Medicine20m1:55-2:15pmHCM organoid models for high-throughput screens**Fabrice Jaffre, PhD / Monash University20m2:15-2:35pmOutcomes of MEKi treatment for cardiac and lymphatic complicationsKatherine Chatfield, MD, PhD / Children's Hospital20m2:35-2:55pmPrecision medicine for RASopathy vascular anomaliesSarah E. Sheppard, MD, PhD, MSTR / NICHD20m2:55-3:15pmESI Selected AbstractTBD10m3:15-3:25pm	Lunch and Poster Session Redux		1h30m	12:15pm
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Noonan syndrome HCM organoid models for high-throughput screens Outcomes of MEKi treatment for cardiac and lymphatic complications Precision medicine for RASopathy vascular anomalies ESI Selected Abstract **Fabrice Jaffre, PhD / Monash University **Fabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Fabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Tabrice Jaffre, PhD / Monash University **Colorado Precision Medicine for RASopathy vascular anomalies **Tabrice Jaffre, PhD / Monash University **Tabrice Jaffr	Family perspective	Tammy Bowers / Noonan Syndrome Foundation	10m	1:45-1:55pm
Outcomes of MEKi treatment for cardiac and lymphatic complications Precision medicine for RASopathy vascular anomalies ESI Selected Abstract Katherine Chatfield, MD, PhD / Children's Hospital 20m 2:35-2:55pm Colorado 2:35-2:55pm 2	Novel mechanisms of PTPN11 signaling in Noonan syndrome	Anton Bennett, PhD / Yale School of Medicine	20m	1:55-2:15pm
lymphatic complications Colorado 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	HCM organoid models for high-throughput screens	**Fabrice Jaffre, PhD / Monash University	20m	2:15-2:35pm
Precision medicine for RASopathy vascular anomalies Sarah E. Sheppard, MD, PhD, MSTR / NICHD 20m 2:55-3:15pm TBD 10m 3:15-3:25pm	Outcomes of MEKi treatment for cardiac and lymphatic complications	•	20m	2:35-2:55pm
	Precision medicine for RASopathy vascular anomalies	Sarah E. Sheppard, MD, PhD, MSTR / NICHD	20m	2:55-3:15pm
Break 30m 3:25-3:55pm	ESI Selected Abstract	TBD	10m	3:15-3:25pm
	Break		30m	3:25-3:55pm

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IV-RASopathies Genetics		·	·		
Moderator: Karen Gripp, MD / Nemours					
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 RASopathic	es 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		
Free Time		1h15	5:15-6:30pm		
Networking Dinner		2h	6:30-8:30pm		

SUNDAY Breakfast		1h	7:00am
V-Predisposition to Malignancy			
Moderator: Marielle Yohe, MD, PhD / NO	CI		
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 / UCDavis	20m	9:30-9:50am
ESI Selected Abstract	TBD	10m	9:50-10:00am
Break		15m	10:00-10:15am
VI-Therapeutic Pipeline			
Moderator: Pau Castel, PhD / NYU Lang	gone		
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