

Third International Meeting on Genetic Syndromes of the Ras/MAPK Pathway: Towards a Therapeutic Approach

August 2-4, 2013
Renaissance Orlando at SeaWorld

DRAFT AGENDA

FRIDAY ~ August 2, 2013		
20:00 – 22:00	Dessert and Poster Session For Symposium Attendees and Advocacy/Family Support Groups	
SATURDAY August 3, 2013		
7:00 – 8:15	Breakfast	
8:15 – 8:30	Welcoming Comments and Introduction	Bruce Korf , University of Alabama, Birmingham and Alcino Silva , University of California, Los Angeles
8:30 – 9:00	Keynote Presentation: TRND Program at NIH and possibilities for drug discovery in the Ras/MAPK disorders	Sittampalam Gurusingham , NCATS
9:00 – 9:30	Advocates' Panel	Advocates Representing CFC, Costello, Neurofibromatosis type 1 and Noonan syndromes
9:30 – 10:50	Ras/MAPK Pathway Phenotypes and Therapeutic Goals Moderators: Karen Gripp , DuPont Hospital for Children and Judith Allanson , Children's Hospital, Eastern Ontario	
	Therapeutic endpoints in NF1	Scott Plotkin Massachusetts General Hospital
	Cardio-facio-cutaneous syndrome	Emma Burkitt Wright University of Manchester
	Costello syndrome	Bronwyn Kerr University of Manchester
	Noonan syndrome	Martin Zenker University of Erlangen
10:50 – 11:10	Break	
11:10 – 12:30	Genomic Approaches Moderator: Ludwine Messiaen , University of Alabama, Birmingham	
	Molecular analysis of NF1	Ludwine Messiaen , University of Alabama, Birmingham
	Structural and functional characterization of RAS mutants	Reza Ahmadian , Heinrich-Heine University
	Use of antisense morpholino oligomers as a gene-therapeutic approach to restore normal splicing	Conxi Lazaro , IMPPC Barcelona
	Ras pathway gene mutations in human genetic disorders	Yoko Aoki , Tohoku University
12:30 – 13:35	Lunch	
13:35 – 14:20	Presentation of Submitted Abstracts by Young Investigators Moderators: Bruce Korf , University of Alabama, Birmingham and Alcino Silva , University of California, Los Angeles	

SATURDAY ~ August 3, 2013 - Continued	
14:20 – 15:40	Ras Pathway Biology and Identification of Therapeutic Targets Moderators: Ype Elgersma , Erasmus University and Benjamin Neel , Ontario Cancer Institute
	Mechanism and treatment for the cognitive deficits associated with animal models of Noonan syndrome Alcino Silva , UCLA
	Modeling RASopathies with hiPSC Sonia Mulero-Navarro , Mt. Sinai School of Medicine
	Learning difficulties in the RASopathies: Pathogenesis and treatment Ype Elgersma , Erasmus University
15:40 – 16:00	Break
16:00 – 17:20	Preclinical Drug Development and Testing Moderator: Bruce Gelb , Mt. Sinai School of Medicine
	Therapeutic approaches to HCM in Noonan syndrome with multiple lentiginos Maria Kontaridis , Harvard Medical School
	Mouse models of Noonan syndrome and other RASopathies Benjamin Neel , Ontario Inst. Can. Res.
	Drug screening for Noonan syndrome with Drosophila Bruce Gelb , Mt. Sinai School of Medicine
18:30 – 20:00	Dinner with a Family Support Group – Participants will be encouraged to partake in the concurrent family forum dinners

SUNDAY August 4, 2013	
7:00 – 8:00	Breakfast
8:00 – 9:20	Clinical Trials Moderator: Katherine Rauen , University of California, San Francisco
	NF Clinical Trials Consortium Bruce Korf , University of Alabama, Birmingham
	Clinical trials for Fragile X syndrome Reymundo Lozano , University of California, Davis
	Use of Raf inhibitors for treatment of melanoma Chao Zhang , Plexxicon
	The NS clinical trial Jesse Gu , Novartis
9:20 – 10:20	Infrastructure to Support Therapeutic Development Moderator: Amy Roberts , Children’s Hospital, Boston
	The CTF therapeutic development pipeline Annette Bakker , Children’s Tumor Foundation
	Tissue banking in pediatric oncology Corrine Linardic , Duke University School of Medicine
	Patient-driven disease registries Pat Furlong , Parent Project Muscular Dystrophy
10:20 – 10:35	Break
10:35 – 12:15	Break-out Sessions with Advocacy Groups
12:15 – 12:30	Final Comments and Discussion Bruce Korf , University of Alabama, Birmingham Alcino Silva , University of California, Los Angeles and Lisa Schoyer , RASopathies Network USA