

Pediatric Translational Research Workshop for Basic Scientists

October 14 – 18, 2013

Colket Translational Research Building • Philadelphia, Pennsylvania

Organizing Committee:

Tom Curran, PhD, *Lead Workshop Organizer*

Peter Adamson, MD

Marni Falk, MD

Rudy Fuentes, PhD

Mary Leonard, MD, MSCE

Yael Mosse, MD

Adam Resnick, PhD

David Taylor, PhD

Wendy Williams, PhD

Fraser Wright, PhD

Athena Zuppa, MD, MSCE

Monday, October 14, 2013 - What is Translational Research?

Chair: Tom Curran, PhD, FRS

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Check In and Breakfast		CTRB 3040
9:00am – 9:30am	Welcome and Introduction	Tom Curran, PhD, FRS , Deputy Scientific Director, CHOP Research Institute, Professor of Pathology and Laboratory Medicine, Cell & Developmental Biology, Perelman School of Medicine, Associate Director, Translational Genomics, University of Pennsylvania	CTRB 3040
9:30am – 10:00am	Participant Introductions	Tom Curran, PhD, FRS (Facilitator)	CTRB 3040
10:00am – 11:00am	Tales From The Clinic – Why Research Matters	Philip Johnson, MD , Chief Scientific Officer and Executive Vice President, Director, CHOP Research Institute, Professor of Pediatrics, University of Pennsylvania	CTRB 3040
11:00am – 11:15am	Break		
11:15am – 12:15pm	Implementation and Dissemination Science: Understanding How to Improve Care	Louis Bell, MD , Chief, Division of General Pediatrics, Associate Chair for Clinical Activities, Attending Physician, General Pediatrics and Infectious Diseases Services, The Children's Hospital of Philadelphia	CTRB 3040
12:15pm – 1:30pm	Mechanism-Directed Therapies for Cystic Fibrosis: Bench to Bedside to Reality Patient Visit and Discussion – Cystic Fibrosis Working Lunch	Ron Rubenstein, MD, PhD , Director, CHOP Cystic Fibrosis Center and Associate Professor of Pediatrics	CTRB 3040

Monday, October 14, 2013 (Continued...)

1:30pm – 2:30pm	Rotavirus Vaccines: From Bench to Market	Paul Offit, MD , Chief, Division of Infectious Diseases, Director, Vaccine Education Center, The Children’s Hospital of Philadelphia	CTRB 3040
2:30pm – 2:45pm	Relocate to CTRB 1200B (First Floor)		
2:45pm – 4:00pm	Inflammatory Bowel Disease (IBD) Short Talks IBD 101 , Robert Baldassano, MD 2:45pm – 3:00pm IBD and the Gut Microbiome , Lindsey Albenberg, DO 3:00pm – 3:15pm Genomics of Very Early-Onset IBD , Judith Kelsen, MD 3:15pm – 3:30pm MicroRNA Biomarkers for Inflammatory Bowel Disease , Joshua Friedman, MD, PhD 3:30pm – 3:45pm Post-transcriptional Control of Gastrointestinal Gene Expression , Louis Ghanem, MD, PhD 3:45pm – 4:00pm	Robert Baldassano, MD , Director, Center for Pediatric Inflammatory Bowel Disease, Colman Family Chair in Pediatric Inflammatory Bowel Disease, The Children’s Hospital of Philadelphia, Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania Attending Physician Presenters	CTRB 1200B
4:00pm – 5:30pm	Patient Panel – Inflammatory Bowel Disease	Robert Baldassano, MD (Moderator)	CTRB 1200B
5:30pm – 7:30pm	Networking Dinner		CTRB Lobby

Tuesday, October 15, 2013 - Foundations of Translational Research

Chair: Peter Adamson, MD

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Breakfast		CTRB 3040
9:00am – 10:00am	Clinical Research: The Path from Discovery to Treatment	Peter Adamson, MD , Chief, Division of Clinical Pharmacology and Therapeutics, Director, Experimental Therapeutics in Oncology, The Children's Hospital of Philadelphia, Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
10:00am – 11:00am	Can Our Health Care System Afford Expensive New Treatments?	Dan Polsky, PhD , Professor of Medicine, Division of General Internal Medicine, Professor of Health Care Management, The Wharton School, Director of Research, Leonard Davis Institute of Health Economics, University of Pennsylvania	CTRB 3040
11:00am – 11:15am	Break		
11:15am – 12:30pm	Immunotherapy for Neuroblastoma	Dave Barrett, MD, PhD , Assistant Professor of Pediatrics, Blood and Marrow Transplant Section	CTRB 3040
12:30pm – 1:30pm	Lunch		CTRB 3040
1:30pm – 2:30pm	Evaluating the Ethics of Clinical and Translational Research	Steven Joffe, MD, MPH , Emanuel and Robert Hart Associate Professor, Director, Penn Fellowship in Advanced Biomedical Ethics, Department of Medical Ethics and Health Policy, University of Pennsylvania Perelman School of Medicine	CTRB 3040
2:30pm – 3:30pm	Discovery of Anaplastic Lymphoma Kinase (ALK) Mutations in Patients with Neuroblastoma: Bedside to Bench	Yael Mosse, MD , Attending Physician, The Children's Hospital of Philadelphia, Assistant Professor, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
3:30pm – 4:30pm	CBS News Feature and Discussion	Yael Mosse, MD (Facilitator)	CTRB 3040
4:30pm – 5:00pm	Travel to City Tap House		
5:00pm – 7:30pm	Offsite Happy Hour and Dinner City Tap House (39 th and Walnut Street)		

Wednesday, October 16, 2013 - Human Genetics and Gene Therapy*Chair: Marni Falk, MD*

Time	Description	Speaker/Organizer	Location
8:00am – 8:30am	Breakfast		CTRB 3040
8:30am – 9:30am	Forwarding Genetic Discovery from the Clinics to the Bench	Ian Krantz, MD , Attending Physician, Associate Professor, Perelman School of Medicine at the University of Pennsylvania Nancy Spinner, PhD, FACMG , Evelyn Willing Bromley Endowed Chair in Pathology, Director of Clinical Cytogenomics, Professor of Pathology and Laboratory Medicine, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
9:30am – 11:30am	Patient Visit and Discussion – Cornelia de Lange Syndrome	Ian Krantz, MD , and Nancy Spinner, PhD, FACMG (Facilitators)	CTRB 3040
11:30am – 11:45am	Pick up lunch outside CTRB 3040 Travel to IRB Session (ARC 123C)		
11:45am – 12:00pm	Overview of the Institutional Review Board	Mark Schreiner, MD , Chair, Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia, Associate Professor of Anesthesia and Critical Care, Associate Professor of Pediatrics, University of Pennsylvania School of Medicine Barbara Engel, MD, PhD , Vice Chair, Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia	ARC 123C
12:00pm – 2:30pm	IRB Observation		ARC 123C
2:30pm – 3:00pm	Questions for the CHOP IRB Meeting	Amy Schwarzhoff, BS, CIP , Director, Human Subjects Research, The Children's Hospital of Philadelphia	ARC 124
3:00pm – 3:15pm	Break and Travel to Classroom		
3:15pm – 3:35pm	Conduct of Clinical Trials in the Field of Gene Therapy	Katherine High, MD , William H. Bennett Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania, Howard Hughes Medical Institute Investigator, Director, Center for Cellular and Molecular Therapeutics, The Children's Hospital of Philadelphia	CTRB 3040
3:35pm – 3:55pm	Chemistry, Manufacturing and Controls for Clinical Gene Therapy	Fraser Wright, PhD , Associate Professor of Pathology and Laboratory Medicine, Division of Transplantation Immunology, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040

Wednesday, October 16, 2013 (Continued...)

3:55pm – 4:20pm	Gene Therapy for Leber Congenital Amaurosis	Jean Bennett, MD, PhD , Professor of Ophthalmology, F.M. Kirby Center for Molecular Ophthalmology, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
4:20pm – 4:45pm	Gene Therapy for Hemophilia B	Katherine High, MD	CTRB 3040
4:45pm – 5:30pm	Patient Visit and Discussion	Katherine High, MD, and Fraser Wright, PhD (Facilitators)	CTRB 3040
5:30pm – 7:00pm	Networking Reception and Presentation The PhD Perspective on Translational Research	Nancy Spinner, PhD, FACMG	CTRB Lobby

Thursday, October 17, 2013 - Human Disease and Therapeutics

Chair: Mary Leonard, MD, MSCE

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Breakfast		CTRB 3040
9:00am – 10:00am	Pediatric Neurodegenerative Disease: Drug Discovery to Clinical Trials	David Lynch, MD, PhD , Professor of Neurology and Pediatrics, The Hospital of the University of Pennsylvania, Perelman School of Medicine at the University of Pennsylvania, Director, Friedreich Ataxia Program, The Children's Hospital of Philadelphia	CTRB 3040
10:00am – 11:00am	Perspectives on Pediatric Translational Medicine: Academe, Industry, and FDA	Stephen Spielberg, MD, PhD , Editor-in-Chief, Therapeutic Innovation and Regulatory Science, Journal of the Drug Information Association	CTRB 3040
11:00am – 11:15am	Break		
11:15am – 12:15pm	Practical Aspects of Setting up a Biotech - Faculty Scientist Perspectives , Robert Levy Practical Aspects of Setting up a Biotech - Business Perspectives , Richard Woodward	Robert Levy, MD , William J. Rashkind Endowed Chair in Pediatric Cardiology, Attending Cardiologist, The Children's Hospital of Philadelphia, Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania Richard Woodward, PhD , President, The Business/Technology Interface, LLC, Chief Executive Officer, Vascular Magnetics, Inc.	CTRB 3040
12:15pm – 1:15pm	Lunch		CTRB 3040
1:15pm – 2:15pm	Mitochondrial Disease: Clinical Presentations, Genetic Diagnosis, Mechanisms, and Therapeutic Research Opportunities	Marni Falk, MD , Attending Physician, The Children's Hospital of Philadelphia, Assistant Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
2:15pm – 3:15pm	Patient Visit and Discussion – Mitochondrial Disease	Marni Falk, MD (Facilitator)	CTRB 3040
3:15pm – 3:30pm	Break		
3:30pm – 4:30pm	Why Do Some People Form Two Skeletons?	Fred Kaplan, MD , The Isaac & Rose Nassau Professor of Orthopaedic Molecular Medicine, Chief, Division of Molecular Orthopaedic Medicine, University of Pennsylvania School of Medicine	CTRB 3040
4:30pm – 5:30pm	Patient Visit and Discussion - <i>Fibrodysplasia ossificans progressive</i> (FOP)	Fred Kaplan, MD (Facilitator)	CTRB 3040
5:30pm – 6:00pm	Travel to Landmark Americana		
6:00pm – 8:00pm	Offsite Happy Hour and Dinner Landmark Americana (3333 Market Street)		

Friday, October 18, 2013 - 21st Century Pediatric Medicine

Chair: Athena Zuppa, MD

Time	Description	Speaker/Organizer	
8:00am – 9:00am	Breakfast		CTRB 3040
9:00am – 11:00am	<p>Fetal Surgery for Myelomeningocele: From Concept to Proof of Clinical Efficacy</p> <p>Overview 9:00 – 9:15am</p> <p>Breakout Discussion (New Surgical Procedures - Human Experimentation versus Clinical trial: The role of pre-clinical research; Ethics of fetal therapy; Non-directive counseling and informed consent) 9:15 – 10:00am</p> <p>Prenatal Diagnosis, Patient Selection, and Counseling 10:00 – 10:30am</p> <p>Video Presentation (The Fetal Surgical Operating Room; Prenatal repair of Myelomeningocele) 10:30 – 11:00am</p>	<p>Alan Flake, MD, MSCE, Attending Surgeon, Director, General Surgery Fellowship Program, Director, Center for Fetal Research, Ruth M. and Tristram C. Colket Jr. Endowed Chair, Pediatric Surgery, The Children’s Hospital of Philadelphia, Professor of Surgery, Obstetrics and Gynecology, Perelman School of Medicine at the University of Pennsylvania</p>	CTRB 3040
11:00am – 1:00pm	<p>Translational Research in Neurodevelopmental Disorders: From Bedside to Bench to Interventions in the Classroom (Short Talks)</p> <p>Clinical Presentations of Autism Spectrum Disorders, Judith Miller, PhD 11:00am – 11:20am</p> <p>Genetic underpinnings of autism spectrum disorders, Hakon Hakonarson, MD, PhD 11:20am – 11:40am</p> <p>Lunch Served at 11:40am</p>	<p>Ted Abel, PhD, Brush Family Professor of Biology, Co-Director, Biological Basis of Behavior Program, Editor-in-Chief, Neurobiology of Learning and Memory, Associate Editor, Behavioral Neuroscience, University of Pennsylvania</p> <p>Neurodevelopmental Disorders Speakers/Panelists</p>	CTRB 3040

Friday, October 18, 2013 (Continued...)

11:00am – 1:00pm	<p>Translational Research in Neurodevelopmental Disorders: From Bedside to Bench to Interventions in the Classroom (Continued...)</p> <p>Type Two Translational Research in Autism Intervention: From Ivory Tower to the Real World, David Mandell, ScD 11:40am – 12:00pm</p> <p>Reduced Sociability in Autism: A Biological Dissection Using Mouse Models, Ted Brodtkin, MD 12:00pm – 12:20pm</p> <p>Modeling Autism Spectrum Disorder using <i>Drosophila</i>, Thomas Jongens, PhD 12:20pm – 12:40pm</p> <p>Neurodevelopmental Disorders Panel Discussion 12:40pm – 1:00pm</p>		
1:00pm – 2:00pm	Unraveling the Genetic Basis of Congenital Heart Defects: How and Why	Elizabeth Goldmuntz, MD , Professor of Pediatrics, University of Pennsylvania Perelman School of Medicine, Attending Cardiologist, Division of Cardiology, The Children's Hospital of Philadelphia	CTRB 3040
2:00pm – 2:45pm	The Role of Pharmacology Research in the Care of Critically Ill Children	Athena Zuppa, MD , Attending Physician, Pediatric Critical Care Medicine, Associate Director of PICU Fellowship Program, The Children's Hospital of Philadelphia, Associate Professor of Anesthesiology, Critical Care and Pediatrics, Perelman School of Medicine at the University of Pennsylvania	CTRB 3040
2:45pm – 3:00pm	Travel to Pediatric Intensive Care Unit (PICU)		
3:00pm – 4:00pm	PICU Visit	Athena Zuppa, MD (Facilitator)	PICU, Main Hospital
4:00pm – 4:15pm	Travel Back to Classroom		
4:15pm – 4:45pm	Moderated Discussion of Clinical Experience	Athena Zuppa, MD (Facilitator)	CTRB 3040
4:45pm – 5:00pm	Closing Remarks	Joseph W. St. Geme, III, MD , Professor and Chairman of Pediatrics and Professor of Microbiology, Perelman School of Medicine at the University of Pennsylvania, Physician-in-Chief, The Children's Hospital of Philadelphia	CTRB 3040