

CHOP Translational Research Workshop for Basic Scientists

September 29 - October 2, 2015

Colket Translational Research Building, CTRB 4040 • Philadelphia, Pennsylvania

Organizing Committee:

Tom Curran, PhD, FRS, *Lead Workshop Organizer*
 Colleen Ambron, MBA
 Peter Adamson, MD
 Dennis Durbin, MD, MSCE
 Marni Falk, MD
 Paulette McRae, PhD

Yael Mosse, MD
 Adam Resnick, PhD
 David Taylor, PhD
 Wendy Williams, PhD
 Athena Zuppa, MD, MSCE

Tuesday, September 29, 2015

Daily Chair: Dennis Durbin, MD, MSCE

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Check In and Breakfast		CTRB 4040
9:00am – 9:45am	Welcome and Overview of Week and Participant Introductions	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 4040
9:45am – 10:45am	Children Are Not Small Adults: An Introduction to Pediatric Translational Research	Dennis Durbin, MD, MSCE , Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania, Director, Office of Clinical and Translational Research, The Children's Hospital of Philadelphia Research Institute	CTRB 4040
10:45am – 11:00am	Break		
11:00am – 12:00pm	Quality Improvement Science: Closing the Gap between Ideal and Actual 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 4040
12:00pm – 1:15pm	Lunch		CTRB 4040
1:15pm – 1:45pm	Introduction to the IRB	Barbara Engel, MD, PhD , Chair, The Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia Amy Schwarzhoff, MBA, CIP , Director, Human Subjects Research, The Children's Hospital of Philadelphia	CTRB 4040
1:45pm – 2:45pm	Rare Disease, Translational Research, and a Basic Scientist	Eileen Shore, PhD , Cali and Weldon Research Professor in FOP, University of Pennsylvania	CTRB 4040

2:45pm – 3:00pm	Break		
3:00pm – 4:00pm	Example of Bench to Bedside: SLE Lupus Nephritis	Kathleen Sullivan, MD, PhD , Chief, Division of Allergy and Immunology, The Children's Hospital of Philadelphia	CTRB 4040
4:15pm – 5:15pm	Academic and Industry Collaboration in Rare Disease Research – The Alexion Perspective	Stephen Uden, MB, BS, MRCP , Senior Vice President of Research, Alexion Pharmaceuticals	CTRB 4040
5:15pm – 6:15pm	Opening Networking Reception		ARC Lobby

Wednesday, September 30, 2015

Daily Chair: Adam Resnick, PhD

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Breakfast		CTRB 4040
9:00am – 10:00am	Mechanism-Directed Therapies for Cystic Fibrosis: Bench to Bedside to Reality	Ron Rubenstein, MD, PhD , Director, CHOP Cystic Fibrosis Center and Associate Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania	CTRB 4040
10:00am – 10:30am	Patient Visit and Discussion – Cystic Fibrosis: Lauren Brenneman	Ron Rubenstein, MD, PhD (Facilitator)	CTRB 4040
10:30am-10:45am	Break and Travel to Main Hospital		
10:45am – 12:15pm		Clinic Visits	Main Hospital
12:15pm-12:30pm	Break and Travel to Networking Lunch		
12:30pm – 2:00pm	Networking Lunch	Faculty/Scientist Roundtable Participants Tom Curran, PhD, FRS; Matthew Maltese, PhD; Eileen Shore, PhD; Gene Griffin, DVM; Adam Resnick, PhD	CTRB 1200B
2:00pm – 2:15pm	Break and Return to Classroom		
2:15pm – 3:15pm	Extracorporeal Support of the Preterm Infant	Marcus Davey, PhD , Research Assistant Professor, The Center for Fetal Research at The Children's Hospital of Philadelphia	CTRB 4040
3:15pm-4:15pm	In Utero Hematopoietic Stem Cell Transplantation	William H. Peranteau, MD , Attending Surgeon, Division of Pediatric General, Thoracic and Fetal Surgery, The Children's Hospital of Philadelphia, Assistant Professor of Surgery, Perelman School of Medicine at the University of Pennsylvania	CTRB 4040
4:15pm – 5:15pm	Data Transparency Through the Lens of Scientific Credibility and the Future of Therapeutics	Stephen Spielberg, MD, PhD , Editor-in-Chief, Therapeutic Innovation and Regulatory Science, The Journal of the Drug Information Association	CTRB 4040

Thursday, October 1, 2015*Daily Chair: Marni Falk, MD*

Time	Description	Speaker/Organizer	Location
8:00am – 9:00am	Breakfast		CTRB 4040
9:00am – 10:00am	Ethics of Clinical Research vs Ethics of Clinical Practice: A Comparison	Chris Feudtner, MD, PhD, MPH , Director, Department of Medical Ethics, Stephen D. Handler Endowed Chair of Medical Ethics, Attending Physician & Research Director, PACT & ICS, The Children's Hospital of Philadelphia, Professor of Pediatrics, Medical Ethics and Health Policy, Perelman School of Medicine, University of Pennsylvania, Associate Division Chief, Division of General Pediatrics, Director, Academic General Pediatrics Fellowship Program	CTRB 4040
10:00am – 11:00am	Mitochondrial Disease: Overview and Translational Research Opportunities	Marni Falk, MD , Assistant Professor of Pediatrics, Division of Human Genetics, The Children's Hospital of Philadelphia, University of Pennsylvania Perelman School of Medicine	CTRB 4040
11:00am – 12:00pm	Patient Visit and Discussion – Mitochondrial Disease	Marni Falk, MD (Facilitator)	CTRB 4040
12:00pm – 1:00pm	Lunch		CTRB 4040
1:00pm – 2:00pm	How Can Clinical Pharmacology Improve the Way We Care for Critically Ill Children?	Athena Zuppa, MD , Associate Professor of Anesthesiology and Critical Care at the Hospital of the University of Pennsylvania, Attending Physician, Associate Director of PICU Fellowship Program, Director, Center for Clinical Pharmacology, Director, Bioanalytical Core Division of Pharmacology and Therapeutics, The Children's Hospital of Philadelphia	CTRB 4040
2:00pm – 2:15pm	Break		
2:15pm – 3:45pm	Mock IRB	Barbara Engel, MD, PhD , Chair, The Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia Mark S. Schreiner, MD , Associate Professor of Anesthesiology and Critical Care at The Children's Hospital of Philadelphia, Perelman School of Medicine at the University of Pennsylvania, Executive Vice-Chair, The Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia Edward Attiyeh, MD , Assistant Professor of Pediatrics, Perelman School of Medicine at the University of Pennsylvania, Division of Oncology, The Children's Hospital of Philadelphia, Vice-Chair, The Committees for the Protection of Human Subjects (IRB), The Children's Hospital of Philadelphia	CTRB 4040

		Amy Schwarzhoff, MBA, CIP , Director, Human Subjects Research, The Children's Hospital of Philadelphia	
3:45pm – 4:00pm	Break		
4:00pm-6:00pm	Ellen Hyman-Browne Memorial Lecture: "Is it Time to Reconsider BRCA1/2 Testing in Children?"	Angela R. Bradbury, MD , Assistant Professor of Medicine at the Hospital of the University of Pennsylvania, Department of Medicine, Hematology/Oncology, Department of Medical Ethics and Health Policy, Perelman School of Medicine at the University of Pennsylvania	CTRB 1200B
	Reception		CTRB Lobby

Friday, October 2, 2015

Daily Chair: Tom Curran, PhD, FRS

Time	Description	Speaker/Organizer	
8:00am – 9:00am	Breakfast		ARC 123ABC
9:00am – 10:00am	An Overview of Autism and Spectrum Disorders: Clinical and Biological Considerations	Robert T. Schultz, PhD , Director, Center for Autism Research, The Children's Hospital of Philadelphia RAC Professor of Psychology and Cognitive Neuroscience, Departments of Pediatrics and Psychiatry, University of Pennsylvania	ARC 123ABC
10:00am – 11:00am	Panel Discussion	Robert T. Schultz, PhD (Facilitator) Panelists: Debra Dunn Kevin Fretz Anna Perng Robert Schmus Amy Kelly	ARC 123ABC
11:00am – 11:15am	Break		
11:15am – 12:15pm	Keynote Address: Translational Science and Drug Development	Garret A. FitzGerald, MD, FRS , Professor of Medicine and Pharmacology, McNeil Professor in Translational Medicine and Therapeutics, Chair, Department of Systems Pharmacology and Translational Therapeutics, Director, Institute for Translational Medicine & Therapeutics, Perelman School of Medicine, University of Pennsylvania	ARC 123ABC
12:15pm-12:30pm	Break		
12:30pm – 1:30pm	Cellular Immunotherapy for Leukemia	Shannon Maude, MD, PhD , Attending Physician, Division of Oncology, The Children's Hospital of Philadelphia, Assistant Professor of Pediatrics, University of Pennsylvania Perelman School of Medicine	ARC 123ABC
	Working Lunch		

1:30pm – 2:30pm	<p>The Human Microbiome: Methods and Applications</p> <p>Genes, Microbiome, Diet and IBD</p>	<p>Frederic Bushman, PhD, W. M. Measey Professor, Chair, Department of Microbiology, Co-PI, PennCHOP Microbiome Program, Perelman School of Medicine, University of Pennsylvania</p> <p>Judith Kelsen, MD, Assistant Professor of Pediatrics, Division of GI, Hepatology, and Nutrition, The Children's Hospital of Philadelphia, Perelman School of Medicine at the University of Pennsylvania</p>	ARC 123ABC
2:30pm – 3:30pm	Microbiome Panel	<p>Panelists</p> <p>Frederic Bushman, PhD</p> <p>Judith Kelsen, MD</p> <p>Lindsey Albenberg, DO, Assistant Professor of Pediatrics, Division of Gastroenterology, Hepatology, and Nutrition, The Children's Hospital of Philadelphia, The University of Pennsylvania</p>	ARC 123ABC
3:30pm – 4:00pm	Closing/Feedback	<p>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</p>	ARC 123ABC