



The University of Pennsylvania and
The Children's Hospital of Philadelphia *present:*

The 2nd Biannual Cystic Fibrosis Research Retreat

June 10, 2010 • Philadelphia, PA

Invited Speakers:

Dr. Peter Durie

(The Hospital for Sick Children)

Dr. John F. Engelhardt

(University of Iowa)

Dr. Eric Olson

(Vertex Pharmaceuticals)

Speakers:

Dr. Noam A. Cohen

(Otorhinolaryngology)

Dr. Denis Hadjiliadis

(Pulmonary and Critical Care)

Dr. Andrea Kelly

(Pediatrics)

Dr. James L. Kreindler

(Pediatrics)

Dr. Todd Lamitina

(Physiology)

Dr. Robert J. Lee

(Physiology)

Dr. Zhe Lu

(Physiology)

Dr. Ronald C. Rubenstein

(Pediatrics)

Dr. Michael S. Stalvey

(UMass)

Chairs:

Dr. J. Kevin Foskett

(Physiology)

Dr. Maria P. Limberis

(Pathology & Laboratory Medicine)

Dr. Maria Mascarenhas

(Pediatrics)

Dr. James M. Wilson

(Pathology & Laboratory Medicine)

Organizers:

Maria P. Limberis & Robert J. Lee

Topics include:

Airway ion transport

Advances in CF animal models

CF-related diabetes

Atypical CF

Adult lung transplantation

Advances in pediatric and adult CF care

Current status of clinical trials

NEW: *Oral presentation of top 3 abstracts*

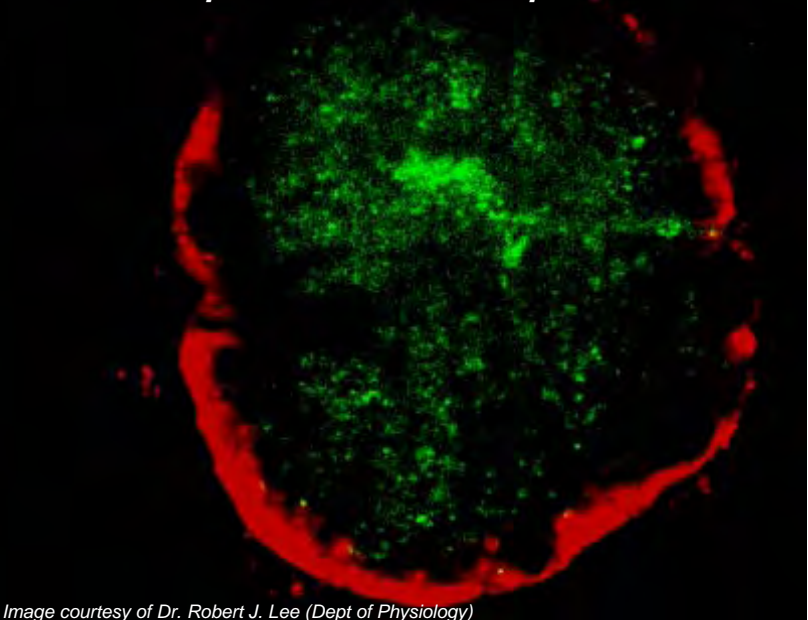


Image courtesy of Dr. Robert J. Lee (Dept of Physiology)

**Deadline for Abstract submission and
Registration is May 24th, 2010.**

Attendance at this meeting is **FREE** upon registration. For more information please contact us at: CFretr10@mail.med.upenn.edu

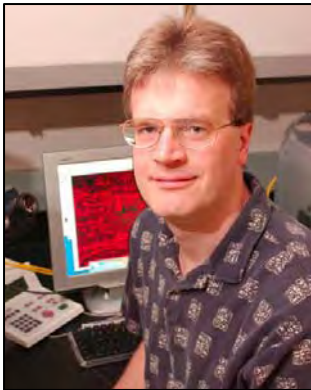
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The *Opening Lecture* "**Phenotypic Characterization of a CFTR Knockout Ferret Model**" will be presented by **Dr. John F. Engelhardt**, Director of the Center for Gene Therapy, Professor and Head of the Department of Anatomy and Cell Biology, and Professor of Internal Medicine, and Roy J. Carver Chair in Molecular Medicine at the University of Iowa Roy J. and Lucille A. Carver College of Medicine. Dr. Engelhardt's research focuses on the study of lung molecular and cellular biology as it relates to the pathogenesis and treatment of cystic fibrosis lung disease, as well as dissecting the pathogenesis of lung disease in animal models of cystic fibrosis.

The *Keynote Speaker* is **Dr. Peter Durie**, Professor of Pediatrics at the University of Toronto who will present on "**What is Cystic Fibrosis?**". Dr. Durie is a Senior Scientist in the program for Physiology and Experimental Medicine, Director of Cystic Fibrosis Research, and Staff Physician in the Division of Gastroenterology and Nutrition at the Hospital for Sick Children. Dr. Durie is an expert in the diagnosis and care of children with inherited disorders of the exocrine pancreas, including cystic fibrosis, and his research focuses on elucidating the pathophysiological basis of cystic fibrosis, understanding the correlations between CFTR genotype and patient phenotype, and identifying genetic modifiers of cystic fibrosis disease.



The *Closing Lecture* "**The Power of the CF Community: Advancing Investigational CFTR-targeted Therapies**" will be presented by **Dr. Eric Olson**, Vice President and Cystic Fibrosis Franchise Leader at Vertex Pharmaceuticals. In collaboration with Cystic Fibrosis Foundation Therapeutics, Dr. Olson and Vertex have been at the forefront of the discovery and development of potentiators and correctors of mutant CFTR. Dr. Olson has been working on the cystic fibrosis program since joining Vertex in 2001. Prior to that, he worked extensively in the fields of bacterial gene expression and new antibiotic discovery at Warner-Lambert and Upjohn Pharmaceuticals (both now Pfizer).

The deadline for Registration & Abstract Submission is Monday, May 24, 2010.
We strongly encourage the participation of graduate students, post-docs and clinical fellow students in the lunch-time poster session.

Three abstracts will be chosen for oral presentation.
Prizes will be given for the best poster and oral presentations.

Questions? Email CFretr10@mail.med.upenn.edu
For information about **FREE** registration, please visit
<http://www.med.upenn.edu/gtp/education.shtml>

Cystic Fibrosis Research Retreat Program

08:00-08:50 AM ON-SITE REGISTRATION / REFRESHMENTS

08:00-08:50 AM POSTER & EXHIBITS SETUP

08:50-09:00 AM WELCOME Maria P. LIMBERIS, PhD

09:00-09:15 AM **SPECIAL LECTURE: Kathi NOVELLI CLAPHAM**
Fighting for my Life...One Breath at a Time

SESSION 1

Animal Models
CHAIR: James M. WILSON, MD, PhD

09:15-09:20 AM INTRODUCTION

09:20-10:15 AM **OPENING LECTURE: John F. ENGELHARDT, PhD**
Intro: James M. WILSON, MD, PhD
Phenotypic Characterization of a CFTR Knockout Ferret Model

10:15-10:35 AM Noam A. COHEN, MD, PhD
Altered Cilia Beat Frequency in the CF Mouse Model

10:35-10:55 AM Todd LAMITINA, PhD
Genetic dissection of CFTR Quality Control Mechanisms in *C. elegans*

10:55-11:10 AM COFFEE BREAK

SESSION 2

CFTR Cellular Physiology
CHAIR: J. Kevin FOSKETT, PhD

11:10-11:15 AM INTRODUCTION

11:15-11:35 AM James L. KREINDLER, MD
Interleukin-17A: Linking Anion Transport and Innate Immunity in Airway Epithelial Cells

11:35-11:55 AM Zhe LU, MD, PhD
Antioxidant Thiocyanate and Cystic Fibrosis

11:55-12:15 PM Robert J. LEE, PhD
Molecular mechanisms of CFTR-dependent fluid secretion by airway submucosal gland serous cells

SESSION 3

PRESENTATION OF TOP 3 ABSTRACTS
CHAIR: Maria P. LIMBERIS, PhD

12:15-12:20 PM INTRODUCTION

12:20-12:35 PM ABSTRACT #1

12:35-12:50 PM ABSTRACT #2

12:50-01:05 PM ABSTRACT #3

01:05-02:10 PM LUNCH / POSTER SESSION / NETWORKING

02:10-03:05 PM **KEYNOTE LECTURE: Peter DURIE, MD**
Intro: Ronald C. RUBENSTEIN, MD, PhD
What is Cystic Fibrosis?

SESSION 4

Advances in CF Care
CHAIR: Maria Mascarenhas, MBBS

03:05-03:10 PM INTRODUCTION

03:10-03:25 PM Denis HADJILIADIS, MD
Lung Transplantation for Cystic Fibrosis

03:25-03:40 PM Andrea KELLY, MD
Cystic Fibrosis Related Diabetes: An Update

03:40-03:55 PM Michael S. STALVEY, MD
CF Bone Disease: Insights into a Growing Problem

03:55-04:10 PM Ronald C. RUBENSTEIN, MD, PhD
Translation of Novel Therapeutic Strategies for CF into the Clinics

04:10-04:25 PM COFFEE BREAK

04:25-05:20 PM **CLOSING LECTURE: Eric OLSON, MD**
Intro: J. Kevin FOSKETT, PhD
The Power of the CF Community: Advancing Investigational CFTR-targeted Therapies

05:20-05:30 PM Awards Presentation Julian Allen, MD

05:30-05:40 PM CONCLUDING REMARKS James M. WILSON, MD, PhD

05:40-08:00 PM **NETWORKING RECEPTION**