



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

## The Inaugural Cystic Fibrosis Research Retreat

**September 10, 2008**

BRB II/III Auditorium, Philadelphia

### Invited Speakers:

**Dr. Richard Boucher**

(University of North Carolina)

**Dr. Bonnie Ramsey**

(Seattle Children's Hospital)

### Chairs:

**Dr. Claire Mitchell**

(Physiology)

**Dr. Jeff Weiser**

(Microbiology and Pediatrics)

**Dr. Virginia Stallings**

(Pediatrics)

### Organizers:

**Dr. Maria Limberis**

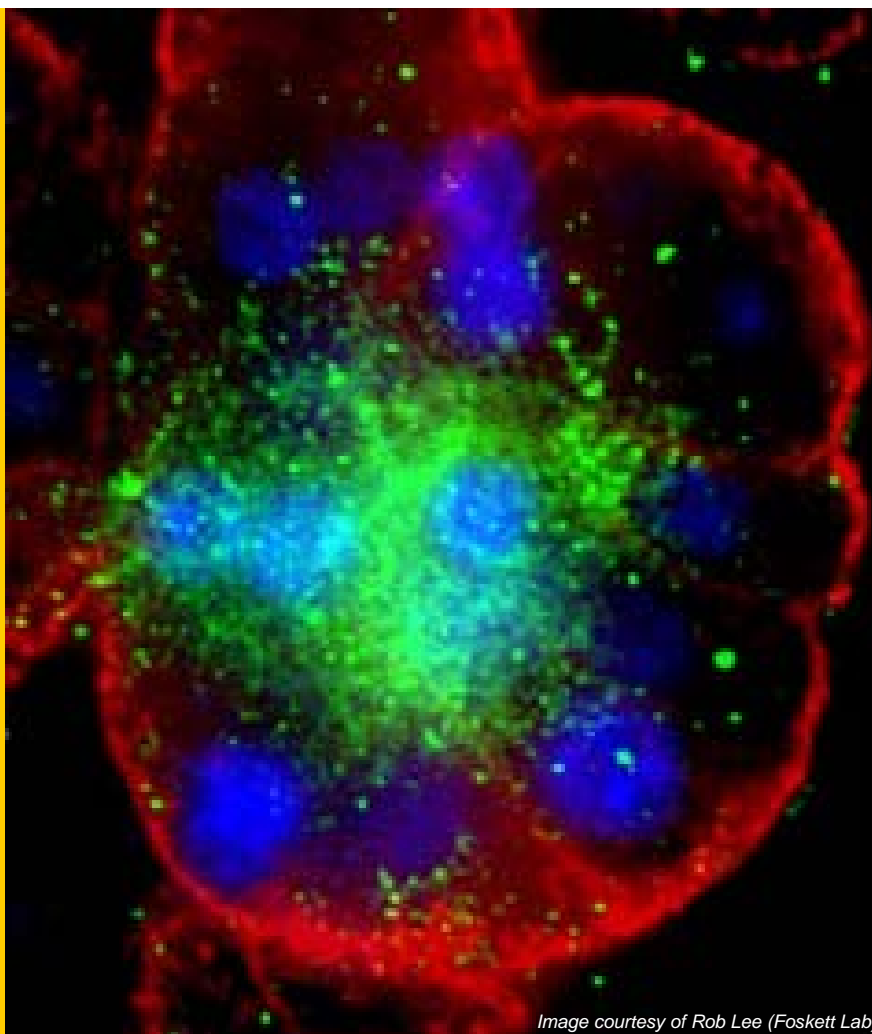
(Pathology & Laboratory Medicine)

**Dr. Kevin Foskett**

(Physiology)

**Dr. Ron Rubenstein**

(Pediatrics)



*Image courtesy of Rob Lee (Foskett Lab)*

**Deadline for Abstract submission and Registration is August 5<sup>th</sup>, 2008.**

Attendance at this meeting is free upon registration. For more information please contact us at:

[CFretr08@mail.med.upenn.edu](mailto:CFretr08@mail.med.upenn.edu)

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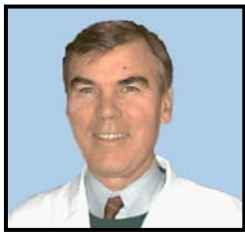


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## The Inaugural Cystic Fibrosis Research Retreat

It is our pleasure to announce *The Inaugural Cystic Fibrosis Research Retreat* organized by the University of Pennsylvania and the Children's Hospital of Philadelphia.

This inaugural one-day retreat will involve a wide array of scientists working in basic science all the way up to clinical development.



Our keynote speaker is **Dr. Richard Boucher** from the University of North Carolina at Chapel Hill. Dr. Boucher is the director of the Cystic Fibrosis/Pulmonary Research and Treatment Center and is world renowned for his contributions to the field of cystic fibrosis from basic science by studying airway physiology, through to the clinical development of pharmaceuticals targeting the basic defect of cystic fibrosis.

The Closing lecture will be given by **Dr. Bonnie Ramsey** from Seattle Children's Hospital. Dr. Ramsey is a pioneer in developing new treatments to meet the needs of the cystic fibrosis community and is the lead investigator of the TOBI™ clinical trials.



We strongly encourage the participation of graduate students, post-docs and clinical fellow students in the lunch time poster session. Monetary poster prizes will be given to the best basic science and the best clinical science posters.

**Abstract Submission & Registration deadline is  
Tuesday, August 5<sup>th</sup>, 2008.**

For any questions please email  
[CFretr08@mail.med.upenn.edu](mailto:CFretr08@mail.med.upenn.edu)

## CF Retreat Agenda, 2008

08:00-09:00 AM **ON-SITE REGISTRATION**

08:00-08:20 AM **POSTER SETUP**

08:00-09:00 AM **BREAKFAST – VIEW POSTERS/EXHIBITS**

09:00-09:05 AM **WELCOME** Maria LIMBERIS, PhD (Pathology & Laboratory Medicine)

09:05-09:15 AM Myra CORCORAN (CFF PA Chapter)  
**CF from a parent's perspective**

### SESSION 1

**CFTR Cellular Physiology**  
*CHAIR: Claire MITCHELL (Physiology)*

09:15-09:20 AM **INTRODUCTION**

09:20-09:40 AM Kevin FOSKETT, PhD (Physiology)  
**Molecular mechanisms of ion and fluid secretion by lung submucosal glands**

09:40-10:00 AM James KREINDLER, MD (Pediatrics)  
**Augmentation of bicarbonate secretion in human bronchial epithelial cells by pro-inflammatory cytokines**

10:00-10:20 AM Noam COHEN, MD, PhD (Otorhinolaryngology)  
**Cilia function in CF mouse nasal epithelium**

10:20-10:40 AM Zhe LU, MD, PhD (Physiology)  
**Membrane phospholipids and CFTR function**

10:40-11:00 AM Ronald RUBENSTEIN, MD, PhD (Pediatrics)  
**Chaperoning CFTR and escorting ENaC**

11:00-11:15 AM **COFFEE BREAK**

### SESSION 2

**Animal Models and New Therapies**  
*CHAIR: Jeff WEISER, PhD (Microbiology)*

11:15-11:20 AM **INTRODUCTION**

11:20-11:40 AM Maria LIMBERIS, PhD (Pathology & Laboratory Medicine)  
**Viral mediated gene therapy for CF**

11:40-12:00 PM Robert LEVY, MD (Pediatrics)  
**Strategies for local Delivery of gene therapy for CF with biodegradable nanoparticles**

12:00-12:20 PM Todd LAMITINA, PhD (Physiology)  
**A *C. elegans* model for  $\Delta F508$  CFTR processing**

12:20-12:40 PM Scott DIAMOND, PhD (Bioengineering)  
**Cationic steroid formulations for viral delivery to lung**

12:40-01:30 PM **LUNCH / POSTER SESSION, NETWORKING**

01:30-02:30 PM **KEYNOTE LECTURE**

Richard BOUCHER, MD (University of North Carolina @ Chapel Hill)  
**Airway surface dehydration in CF: Role in pathogenesis and biomarker for development of therapeutics**

### SESSION 3

**Advances in CF Care**  
*CHAIR: Virginia STALLINGS, MD (Pediatrics)*

02:30-02:35 PM **INTRODUCTION**

02:35-02:50 PM Suzanne BECK, MD (Clinical Pediatrics)  
**Quality improvement in CF: Improving outcomes**

02:50-03:05 PM Denis HADJILIADIS, MD (Pulmonary, Allergy & Critical Care Division)  
**Advances in lung transplantation**

03:05-03:15 PM **COFFEE BREAK**

03:15-03:35 PM Richard FINKEL, MD (Pediatrics and Neurology)  
**PTC124 promotes read through of premature termination codon mutations in Duchenne Muscular Dystrophy**

03:35-03:55 PM David LIPSON, MD (GSK)  
**CF and the pharmaceutical industry**

03:55-04:15 PM Susan LINDEE, PhD (History and Sociology of Science)  
**The Uncertainty Gene: CF in social and political context**

04:15-05:15 PM **CLOSING LECTURE**

Bonnie RAMSEY, MD (Seattle Children's Hospital)  
**Anatomy of a clinical trial in CF: How the TDN has impacted on this process**

05:15-05:25 PM **CONCLUDING REMARKS** James M. WILSON, MD, PhD (Pathology & Laboratory Medicine)

05:25-05:35 PM **AWARD PRESENTATIONS** Kevin FOSKETT & Ronald RUBENSTEIN

05:35-09:00 PM **DINNER RECEPTION (14th Floor BRB Faculty Lounge)**