Cystic Fibrosis Center of Excellence

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Dear friends, patients, families, and colleagues,

As the crazy year that was 2016 draws to a close, we write to send you the latest edition of our e-newsletter. As always, there is much to report on in the following pages. Our main focus for this e-newsletter is reporting on the 2016 North American Cystic Fibrosis Conference, held this past October in Orlando, FL. The CF@LANTA team was very well represented at the conference, which is the largest gathering of scientists, physicians, and others focused on CF in the world. CF@LANTA members presented their research progress, led symposia and workshops, and even presented one of the plenary sessions. See the following pages for a list of ways in which we contributed to the conference.

In addition, we share the news that our own Dr. Daniel Caplan will retire in February, 2017. Dr. Caplan is well known as the “grandfather of CF” in Atlanta, and is much loved by his patients, for whom he has provided care for 48 years. We plan to celebrate his life, his commitment to CF patients, and his contributions to our CF Program at the annual Dr. Dan Caplan CF Family Science Dinner, in the Spring. Please be on the lookout for details, to be announced in the coming months.

Finally, we report on the recent book club that we held on November 30. For the second year in a row, we gathered together to discuss a book that has ties to science and the role of science in the world. This year’s book, “Brave Genius: A Scientist, a Philosopher, and Their Daring Adventures from the French Resistance to the Nobel Prize,” by Sean Carroll, was recommended by Dr. Rabin Tirouvanziam, Assistant Professor of Pediatrics. The book describes the great contributions of Albert Camus and Jacques Monod, both Frenchman, during the invasion of France by Germany in World War II, and then the important work of both men following the war: giants in philosophy and molecular biology, respectively. Camus was awarded the Nobel Prize in Literature in 1957, and Monod shared the Nobel Prize in Physiology or Medicine with two other colleagues in 1965. A mixture of discussion of the fight for justice and the scientific search for the fundamental nature of life was shared by about ten team members, over (of course) French wine and cheese.

We hope that each of you has a wonderful holiday season, and are able to spend quiet time with family and friends. As we enter a new year, we recall the many blessings that we share—great friends, great colleagues, great excitement for what lies ahead in 2017. Those of us who are healthy will remember those whose health is threatened by CF, asthma, and many other diseases, and we will become even further motivated to do all we can on behalf of our patients—those we know and the many who count on us for their futures.

With best wishes,
Nael A. McCarty, PhD, CF@LANTA
The 2016 North American Cystic Fibrosis Conference was held in Orlando, Florida. CF@LANTA was represented at all levels of presentations, sharing all of the great work happening in our center.

Posters

- Cui, G.; Khazanov, N.; Imhoff, B.; Senderowitz, H.; McCarty, N.A. NOVEL POTENTIATORS OF CYSTIC FIBROSIS TRANSMEMBRANE CONDUCTANCE REGULATOR IDENTIFIED WITH PHARMACOPHORE MODELING AND EXPERIMENTAL SCREENING
- Goeckeler-Fried, J.; Estabrooks, S.K.; Chiang, A.; Chung, W.; Ye, Z.; Denny, R.A.; Weissman, A.M.; Camacho, C.J.; Sorscher, E.J.; Brodsky, J.L. IDENTIFICATION OF NOVEL INHIBITORS OF UBIQUITINATION THAT IMPROVE RESCUE OF F508DEL-CFTR BY VX-809
- Oliver, K.E.; Wang, W.; Wolpert, M.J.; Maya, J.; Icyuz, M.; Hong, J.S.; Hartman, J.L.; Sorscher, E.J. RIBOSOMAL TARGETING CORRECTS CFTR CLASS I AND II DEFECTS
- Chung, W.; Fortenberry, J.A.; Sokirny, I.; Wu, J.; Chiang, A.; Goeckeler-Fried, J.; Sterner, D.; Mattern, M.; Brodsky, J.L.; Sorscher, E.J.; Kumar, S. AUGMENTATION OF THE AVAILABLE POOL OF CFTR FOR SMALL MOLECULE CORRECTION BY INHIBITING THE E3 LIGASE, GP78
- Day, B.J.; Min, E.; Nichols, D.; Chandler, J.D. BACTERIAL THIOREDOXIN REDUCTASE IS A TARGET FOR HYPOTHIOCYANITE-MEDIATED ANTIMICROBIAL EFFECTS
- Stauffer, B.; Cui, G.; Infield, D.T.; McCarty, N.A. S. AUREUS SPHINGOMYELINASE IS A STATE-DEPENDENT INHIBITOR OF CFTR
- Beaty, T.C.; Brown, M.R.; Forrest, O.A.; Ingersoll, S.; Stecenko, A.; Tirouvanziam, R. EFFECT OF INHALED CORTICOSTEROID THERAPY WITHDRAWAL ON CF LUNG FUNCTION AND INFLAMMATORY MARKERS
- Bedi, B.; Yuan, Z.; Varga, J.J.; Wei, J.W.; Goldberg, J.B.; Stecenko, A.; Sadikot, R.T. PSEUDOMONAS AERUGINOSA ISOLATES FROM PATIENTS WITH CYSTIC FIBROSIS INHIBIT PPARγ EXPRESSION IN MACROPHAGES AND EPITHELIAL CELLS
- Rada, B.; Floyd, M.; Yoo, D.; Cullen, C. SWIMMING MOTILITY MEDIATES NET FORMATION STIMULATED BY FLAGELLATED PSEUDOMONAS AERUGINOSA
- Molina, S.A.; Vance, R.J.; Imhoff, B.; McCarty, N.A.; Koval, M. ACTIVATION OF AKT SIGNALING IN HUMAN AIRWAY CELLS EXPRESSING MUTANT CFTR DIFFERS COMPARED TO WILD-TYPE
Icyuz, M.; Mutyam, V.; Rowe, S.M.; Sorscher, E.J.; Hartman, J.L. GENOME-WIDE ANALYSIS IN YEAST TO IDENTIFY MOLECULAR TARGETS PROMOTING READTHROUGH


Rab, A.; Wen, H.; Manfredi, C.; Ehrhardt, A.; Joshi, D.; Sorscher, E.J.; Hong, J.S. MODEL SYSTEMS FOR INVESTIGATING MOLECULAR PATHOGENESIS OF RARE CFTR ALLELES

Bihler, H.; Harrington, J.; Musisi, I.; Mercadante, D.; Hong, J.S.; Rab, A.; Sorscher, E.J.; Saltz, J.; Mahiou, J.; Wong, E.; Liang, F.; Mense, M. FUNCTIONAL CHARACTERIZATION OF ESTABLISHED AND NOVEL NONSENSE MUTATION READTHROUGH MODULATORS IN ENGINEERED FRT CELL LINES AND PRIMARY HUMAN BRONCHIAL EPITHELIAL (HBE) CELLS


Zhang, Z.; Ma, H.; Peng, L.; Lai, H.J. GUT MICROBIOTA IN EARLY CHILDHOOD IN CYSTIC FIBROSIS

Yang, J.; Peng, L.; Zhang, Z.; Rahman, A.; Ma, H.; Lai, H.J. JOINT PROFILE OF RESPIRATORY INFECTIONS AND THEIR ASSOCIATIONS WITH BREASTFEEDING IN INFANTS WITH CF

Griego, B.; Tataria, S.; Kirchner, K. INVESTIGATING THE IMPROVEMENT IN PHYSICAL AND PULMONARY ENDURANCE AS MEASURED BY THE MODIFIED SHUTTLE WALK TEST AND FEV1 IN PEDIATRIC PATIENTS ADMITTED TO THE HOSPITAL FOR ACUTE CYSTIC FIBROSIS EXACERBATION

Patel, K.; Britt, E.; Duval, M.A.; Kirchner, K. VITAMIN D SUPPLEMENTATION: HOW MUCH IS ADEQUATE? OUTCOMES FROM A QUALITY IMPROVEMENT PROJECT AT A PEDIATRIC CYSTIC FIBROSIS CENTER

Lindsay, K.A.; Revilla, E.; Tangpricha, V.; Stecenko, A. EFFECTIVENESS OF WEIGHT-BASED, WEEKLY DOSING OF VITAMIN D3 IN CHILDREN WITH CYSTIC FIBROSIS


Hunt, W.R.; Guentert, D.E.; Brown, L.S.; Stecenko, A. PREDIABETES AND REDOX IMBALANCE IN YOUNG CHILDREN WITH CF


Blair, S.D.; Gardner, L. ETHICAL CONSIDERATIONS FOR IMPLEMENTING CAREGIVER MENTAL HEALTH SCREENING WITHIN PEDIATRIC INSTITUTIONS
NACFC Presentations 2016 continued


Lunch Roundtable Leaders
- Dahlman, J.: NEXT GENERATION STRATEGIES FOR CF GENE THERAPY
- Freeman, A.J.: WORKING UP ELEVATED LIVER ENZYMES
- Stephenson, K.: INCREASING CALORIES FOR INFANTS

Workshop Session Chair
- DiMagno, M.J.; Freeman, A.J. Gastroenterology & Hepatology
- Larson-Ode, K.; Tangpricha, V. Endocrine (Research)
- Crews, B.: Mid-level Primary Care
- Pendley, S.: Nurse Mentoring & Education
- Hong, J.: CFTR 2016

Workshop or Symposium
- Blair, S.: MENTAL HEALTH SCREENING
- Freeman, A.J.: GASTROENTEROLOGY & HEPATOLOGY, INTRODUCTION
- Stecenko, A.A.: ROLE OF INSULIN SECRETION & OXIDATIVE STRESS ON THE DEVELOPMENT OF CFRD: A CLINICAL PERSPECTIVE
- Goldberg, J.B.: VACCINES FOR CF INFECTIONS: EPISODE IV - A NEW HOPE

Plenary
- Drumm, M.L.: Sorscher E.J. A CURE FOR ALL: LEAVING NO ONE BEHIND

Selected as one of the top 5 poster presenters
- Chandler, J.
- Plyler, Z.E.

Chair of CFTR Folding Consortium Meeting
- Sorscher, E.J.
New Faculty: Jeong Hong, PhD

Dr. Hong trained as a molecular virologist with a focus on adenovirus fiber protein responsible for binding to cell surface receptor. Her work led to development of strategies to modify cellular tropism of adenovirus and formed the basis for studies of therapeutic viral retargeting by numerous laboratories.

A long standing interest of her laboratory has pursued studies of cystic fibrosis (CF). For more than 20 years, she has directed laboratory aspects of a highly collaborative and productive joint CF research program with Dr. Eric Sorscher, former head of the UAB CF Research Center and now a professor at Emory and a member of CF@LANTA. Her group investigates structural and functional aspects of the CFTR gene product in relation to disease associated mutations, with the goal of promoting new ‘precision-type’ treatments. She has served as a member of the international CF theratyping initiative since its inception in 2014, and developed epithelial cell models encoding clinically relevant CF alleles.

Outside lab, Dr. Hong enjoys gardening and spending time with her family.

~submitted by Jeong Hong

New Faculty: Joon Chung, PhD

Joon Chung is a new faculty of the Center for Cystic Fibrosis and Airways Disease research (CF-AIR). After receiving his PhD and postdoctoral fellowship from the University of Alabama at Birmingham, he had joined the cystic fibrosis research center at UAB where his research had been focused on new approaches to develop drugs that improves rescue of defective CFTR proteins. He joined the Emory University Pediatrics Department as an assistant professor in September 2016. Dr. Chung’s research is focused on understanding mechanisms underlying defects of CFTR mutations and developing experimental therapeutics. He has developed a novel biochemical drug screening method targeting intra molecular domain interactions to improve the chloride channel activity of F508del CFTR. Currently, he is developing a novel class of CFTR modulators (‘amplifiers’) that complement the substrate limitations of other CF therapeutic modalities such as ‘correctors’ and ‘potentiators’. In particular, he is evaluating experimental drugs that target the ubiquitination steps in the protein degradation pathway to augment pools of CFTR mutant proteins for pharmacologic correction.

Outside of his research, he is enjoying a variety of cultural and culinary experiences with his family and learning to navigate busy streets in Atlanta.

~submitted by Joon Chung
Recent CF-AIR Publications


If you have a publication you would like in the next newsletter, email Karen Kennedy, kmurra5@emory.edu.

Recent CF-AIR visitors

Marshall Plaut, PhD, August 30, NIAID Funding opportunities: Research focused on asthma, allergy (including food allergy and atopic dermatitis) and airway biology

Joshua Chandler, PhD, October 11, Targeting small molecules that drive big problems in cystic fibrosis and other inflammatory diseases

Ineke Braakman, PhD, November

Peter van der Sluijs, PhD, November
**Events for Researchers**

These events will be taking a break for the summer months, check the websites below for the upcoming schedule.

Each month there are several opportunities for CF-AIR researchers to get together to discuss their work.

- **CF-AIR Faculty and Trainees Research (CF-TR):**
  On the first Tuesday of the month, faculty chalk talks on either the overall work in their lab, or on a grant proposal planned for submission soon. On the third Tuesday of the month trainee chalk talks discussing planned manuscripts or fellowship proposals. Meet at noon in ECC 302

- **CF-AIR Workshop:**
  A weekly Wednesday meeting for research-in-progress and journal club presentations. Meet at 4:30 pm in ECC 302

- **CF Scholars Meetings:**
  A monthly program for CF Scholars, Friday afternoons, see website schedule

More information and current schedules can be found on

[www.pedsresearch.org/research/centers/cf-air/seminars-workshops/](http://www.pedsresearch.org/research/centers/cf-air/seminars-workshops/)

and


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**Clinics:**

Children’s Healthcare of Atlanta
CF Care Center:
Children’s at North Druid Hills
1605 Chantilly Drive NE
Atlanta, GA 30324
404-785-2000

Children’s at Scottish Rite
Cystic Fibrosis Affiliate Program
5455 Meridian Mark Road, Suite 200
Atlanta GA 30342
404-785-2898

Emory Adult CF Clinic: 404-778-7929

**Website:**

[www.pedsresearch.org/research/centers/cf-air](http://www.pedsresearch.org/research/centers/cf-air)

If you are interested in supporting our research and outreach programs please visit:

[www.pedsresearch.org/research/centers/cf-air/donors-visitors/](http://www.pedsresearch.org/research/centers/cf-air/donors-visitors/)

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