

The Emory+Children's Cystic Fibrosis Center of Excellence

Volume 5, Issue 1

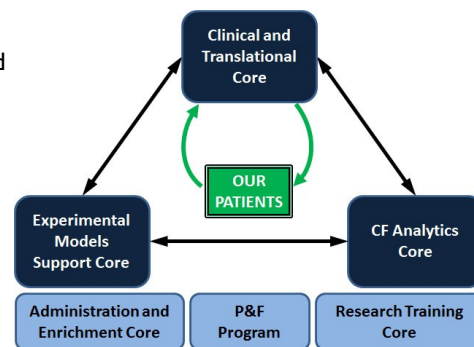
February 2015

Update from the Director

Dear friends of **CF@LANTA**:

It is my great pleasure to present you with this newest edition of our newsletter. Upon reading this, we hope you'll agree that great things are happening for CF in Atlanta!

First, we are happy to announce that we were invited by the CF Foundation to submit an application for support as one of their Research and Development Program (RDP) sites. The RDP mechanism has been in existence since the 1980s – the best CF research centers in the country are supported by one of these grants. Our proposal, submitted February 2, was centered upon infrastructure for translational research targeting CF-related diabetes and acute pulmonary exacerbations, two critical aspects of CF disease progression. As shown in the design figure presented here, the work begins and ends with our patients.



Nael A. McCarty, PhD
Center Director

Other major items in this edition focus on the events surrounding last October's North American CF Conference. Atlanta did us proud as the host city for this event - the largest CF conference in the world. Please see the following pages for details of special events we held before, during, and after the Conference.

Finally, this is an All-Hands-on-Deck call in preparation for the upcoming Great Strides campaign. The Great Strides Walk-a-Thon is the largest fundraising event for the CF Foundation, and the Atlanta walk has broken the national record three years in a row. Let's keep this going! If you're interested in helping the **CF@LANTA** team – perhaps by serving as the group leader for your component at Scottish Rite or Egleston, or in some other way – please contact me right way. The walk is May 16. I'm happy to report that Children's has joined as a "Mission Sponsor" for Great Strides 2015.

-Nael McCarty, PhD

Inside this issue:

Neutrophils mini-symposium	2
Profile: Tim Beaty	
ShamRockin' for a Cure	3
CF-AIR Lab Visit	4
NACFC 2014 Events	5

Recent Visitors

- Calvin Cotton, PhD, October 2014, Case Western Reserve University, *Large-scale expansion of primary airway epithelial cells from CF patients*
- Steve Aller, PhD, October 2014, University of Alabama, Birmingham, *Probing a mechanism of correcting the major CF defect (F508del): is there an actual binding pocket?*
- Stanley Szefer, MD November 2014, Children's Hospital Colorado, *Predicting and Preventing Asthma Exacerbations in Inner City Children*
- Michelle Trivedi, MD, November 2014, MassGeneral Hospital for Children, *Maternal Intake of Dietary Carbon Methyl Donors in Pregnancy and Childhood Asthma at 7 Years*
- Devon Greene, MD, UCOM-Chatanooga/Children's Hospital at Erlanger, February 2015, *Defining asthma with cohort studies and addressing asthma with community and school interventions*

Mini-symposium: Neutrophils in CF: basic and clinical science



Rabin Tirouvanziam, PhD, organizer of the mini-symposium

On the heels of the North American CF Conference, held this year in Atlanta, the Emory+Children's CF-AIR Center convened on October 12-13, 2014, a scientific panel to discuss the critical topic of neutrophils in CF, with an audience of scientists and trainees from the Center and neighboring institutions. Neutrophils are a type of white blood cells that are recruited in large numbers and undergo an abnormal activation process in CF airways, hampering clearance of bacteria and exerting severe damage to the lung. At the initiative of Dr. Rabin Tirouvanziam, Assistant Professor in the Emory+Children's CF-AIR Center, an impressive group of national and international experts on CF inflammation and neutrophil biology gathered for an exciting two-day mini-symposium. These included: Dr. Leo Koenderman, Professor of Immunology at the University of Utrecht in the Netherlands, Dr. Dominik Hartl, Director of the CF Clinic at the University of Tübingen in Germany, Dr.

Jon Koff, Director of the Adult CF Clinic at Yale University, Drs. Amit Gaggar and Zdenek Hel, respectively Associate Professor of Pulmonary and Critical Care Medicine and Assistant Professor of Pathology at the University of Alabama in Birmingham, Dr. Balázs Rada, Assistant Professor of Veterinary Medicine at the University of Georgia in Athens, and Dr. Daniel Kalman, Associate Professor of Pathology at Emory University. In a lively mix of slide presentations and panel discussions, the group discussed recent data demonstrating that neutrophils in blood comprise several functional subsets (Dr. Koenderman) and are extremely adaptable regarding both effector and regulatory functions (Drs. Hartl, Gaggar, Hel, Rada, Kalman and Tirouvanziam). Pushing research boundaries beyond the conventional limits of short-term cultures and animal experiments, several investigators presented novel methods and models for

investigating neutrophil function as it occurs in patients, thus offering exciting opportunities for mechanistic investigation and drug testing (Drs. Koenderman, Hartl, Gaggar and Tirouvanziam). This first-ever mini-symposium on neutrophils in CF has sparked several new collaborative projects among the participants. It will be followed by a similar event organized on the margin of the 2015 European CF Conference in Brussels, Belgium, in June 2015. Thus, the event at Emory kick-started an important collaborative effort around a strong core of like-minded investigators to develop better basic understanding and clinical solutions to efficiently counter neutrophilic inflammation in CF. Our thanks to Greg Duncan (CEO), Bill Reddick (CFO), Eric Springman (CSO) and Ralph Grosswald (VP of Operations) at CeltaSys, an Atlanta-based biotech developing novel treatments for CF (<http://www.celtasys.com>), for their generous sponsorship and help with organizing this event.

New Faculty: Tim Beaty, MD



Tim Beaty, MD

The newest recruit in our quest to better serve CF patients in Atlanta and beyond is Timothy Beaty, MD. Dr. Beaty will complete his Pediatric Pulmonology Fellowship at Emory University and Children's Healthcare of Atlanta and will continue on our team at Emory/Egleston CF Care center on July 1, 2014. Dr. Beaty earned his medical degree from Saint George's University and completed his Pediatric Residency at Tulane University Hospital for Children. It was there that Dr. Beaty discovered the incredible bond between CF physicians

and their parents, a bond that eventually drew him to pursue a career in pediatric pulmonology. "I saw most of my colleagues choose specialties which do not allow for relationships with their patients, like ER and Intensive Care. Although that aspect of healthcare is important, I could not imagine a career of caring for strangers. My desire is to truly become a trusted partner in the lives of my patients. The doctor-patient relationship in CF is the strongest I have seen, and I believe that relationship is a big part of what has led to the

incredible improvement in CF care – I want to be a part of that." In addition to being a care provider in the Emory and Children's CF Care Center, Dr. Beaty will be earning a Masters Degree in Clinical Research from Emory University and will continue his research on CF therapeutics and epidemiology. Outside of work, Dr. Beaty is learning his wife's native language of Japanese, a skill in which his 20 month old toddler has already surpassed him. He also enjoys reading history and fiction and plays guitar for his church's worship band.

ShamRockin' for a Cure Returns March 7, 2015!

Mark your calendars, cancel your plans and bring out your best green so that you won't get pinched – **ShamRockin' for a Cure** (www.shamrockinforacure.com) looks forward to hitting the million-dollar milestone as it returns for its 7th year on Saturday, March 7, 2015!

You've never heard of **ShamRockin' for a Cure**?!! **ShamRockin' for a Cure** is an event benefiting the Cystic Fibrosis Foundation that's putting the FUN back in **FUN**draiser. Unquestionably the definitive St. Paddy's Day Celebration in North Fulton, **ShamRockin' for a Cure** is a premier "party with a purpose" where over 1500 people gather at Verizon Wireless Amphitheater to "eat, drink, and help cure cystic fibrosis." The Shamrockin' Army has raised close to a million dollars in just 6 years, and they rock. With the help of incredible individuals and organizations including Title Sponsor, Georgia's Own Credit Union, **ShamRockin' for a Cure** has

quickly become a tradition for those seeking awesome entertainment, spectacular food and spirits, a little luck in the silent auctions, and a way to help fight cystic fibrosis!

This past November, members of the ShamRockin' Army visited the labs of the Emory+Children's CF Center of Excellence to better understand the research that goes into controlling and ultimately curing CF.

"Today, I finally got to meet the scientists that are pushing the CF boulder up the hill," said Mary Guiney, co-founder of **ShamRockin' for a Cure** while on the tour of the lab. "Before I met these scientists, I was only praying for our CF families. But then I realized that I needed to pray for the scientists too. I needed to start praying for the scientists who are using the funds we were raising. If there's a silver lining to this disease, these magical partnerships are it! Our friends Jake & Gavin Baker, who suffer from Cystic Fibrosis, need their excitement

and enthusiasm; and these incredible scientists need our money."

"Recruiting, top talent, team chemistry - these are common buzz words in the business world. But when it comes to science, these are truly game changers. The CF Center of Excellence NEEDS the best minds in the world wrestling through the mind boggling science that it takes to save Jake & Gavin. Imagine if they know the brilliant scientist who can get them over that last hurdle, but they can't afford to bring them on staff? Imagine that one machine, that one experiment, that one grant - that **ShamRockin' for a Cure** attendees can help them get!!"

Sponsor packages (starting at \$1,500) and individual tickets (~\$85) to the party are on sale now! Go to www.shamrockinforacure.com to learn more!



Pictures from ShamRockin' for a Cure 2014!

CF-AIR Lab Visit



ShamRockin' Army members and CF-AIR Researchers

In November 2014 members from the ShamRockin' Army visited several of the labs at the Emory-Children's Center. This was a chance for family, friends and supporters of CF patients to see the CF-AIR researchers in action and for the CF-AIR researchers to share their work. The visit turned into a chance for the visitors to learn more about the process of

science and all of the steps that go into tackling the disease of CF as well as other diseases. Dr. Arlene Stecenko discussed her clinical work with CF patients as well as how researchers obtain funding for their work through competitive grants. Dr. Mike Koval shared how his research path has changed to become more disease focused as an application of the

exciting basic science discoveries he and his lab has made. Dr. Joanna Goldberg explained how she studies bacteria that may be found in the lungs of CF patients. By understanding the bacteria, Dr. Goldberg's work hopes to aid the treatment of bacterial infections in patients with CF and other diseases. Dr. Tirouvanziam explained how his lab focuses on certain kinds of cells in the body, neutrophils, and how they may be different in CF patients, with an eye to how such knowledge may direct future and current drug treatments. Finally, the day ended with Dr. Nael McCarty showing off his sensitive instrumentation which can directly measure changes in the CFTR protein's electrical characteristics due to mutations found in CF patients.

All in all, the day was a success. A reminder to the researchers that their tiresome work is for people in their community. And hope to the ShamRockin' Army members that their fundraising supports passionate and smart scientists working for them every day.



Lab tours and chatting around breakfast

NACFC 2014 in Atlanta Events

CF Family Science Dinner

To kick off the week of local CF events prior to the NACFC 2014, Children's Healthcare of Atlanta, the Atlanta CFF Chapter, Emory University and CF@LANTA sponsored the first Dr. Daniel Caplan CF Family Science Dinner on October 6, 2014. The night featured talks for CF families to learn the latest science in treating and curing CF. Speakers included both Atlanta-based researchers and international researchers

visiting for NACFC. The speakers were Arlene Stecenko, MD, Seth Walker, MD, Felix Ratjen, MD, PhD, Facundo Fernandez, PhD, and Bob Scholte, PhD. Additionally, Dr. Daniel Caplan was honored for his work in the field. The event included both pre-seminar dinner and a post-seminar reception allowing for the attendees to chat with the speakers and ask questions. The night was a great success!



Mini-symposium on Early CF Lung Disease

On October 7, 2014 CF@LANTA with Children's Healthcare of Atlanta and Emory University hosted a mini-symposium on Early CF Lung Disease. This researcher-focused event brought together researchers from around the globe to discuss their work on this important topic. Felix Ratjen, MD, PhD, Facundo Fernandez, PhD, Sam Moskowitz, MD, Bob Scholte, PhD, and JP Clancy, MD each spoke and answered questions

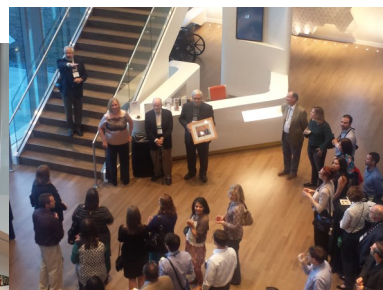
about their work. After the symposium was a Preview of Posters for NACFC. A dozen individuals shared their work in poster form as a preview to their poster presentations at the conference the following weekend. Atlanta-based students, postdocs, fellows and faculty participated, generating conversation in a more relaxed and less busy environment than the impending conference.

Agendas and photo slideshows from each NACFC pre-event can be found on our website at

www.pedsresearch.org/centers/sub-pages/cf-air-cf-education-outreach

CF in GA Cocktail Event

To celebrate the events of NACFC being in Atlanta in 2014, the Emory+Children's CF Care Center hosted an evening at the National Center for Civil and Human Rights on October 10, 2014. This cocktail event did not include a clinical or scientific lecture, and instead was a time for Georgia's CF community to mingle and celebrate all of the clinical and scientific milestones in treating and curing CF that are happening right in Georgia.



Even Augusta-based colleagues were able to join in this Atlanta event for a truly Georgia-wide gathering!



CF-AIR Research Workshop 2015

The CF-AIR Research Workshop meets weekly on Wednesdays at 4 pm in room 302 of the Emory-Children's Center. Each month follows a general pattern unless we have a special visitor or event.

1st week: Journal Club (contact Sarah Ingersoll to sign up)

2nd-4th week: Research Seminar (contact Danny Infield & Brandon Stauffer to sign up)

Upcoming:

February 25, 2015: Danny Infield (McCarty Lab)

March 4, 2015: Arlene Stecenko, MD and Maret Maliniak, MPH

March 11, 2015: Journal Club, Brandon Stauffer (McCarty Lab)

March 19, 2015 (Thursday): Eitan Kerem, MD, Hadassah Hebrew University Medical Center, Israel, "Cystic Fibrosis: The Story of a Journey Through Phenotype, Genotype and Drug Development." (HSRB Auditorium, 11:30 am)

March 25, 2015: Randy Hunt, MD

April 29, 2015: Jason Papin, PhD, University of Virginia (HSRB Auditorium)

Full updated scheduled at:

www.pedsresearch.org/centers/sub-pages/cf-air-seminars-workshops/

Staying in Touch

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/
centers/detail/CF-AIR](http://www.pedsresearch.org/centers/detail/CF-AIR)

If you are interested in supporting our research and outreach programs please visit: www.pedsresearch.org/centers/sub-pages/cf-air-donors-visitors/

Contact:

Nael McCarty, PhD: Program Director
namccar@emory.edu

Karen Kennedy, Program Coordinator
kmurra5@emory.edu

