CF-AIR & CF@LANTA NEWSLETTER

Cystic Fibrosis Center of Excellence

Emory University - Children's Healthcare of Atlanta - Georgia Tech - Augusta Univ. - Georgia State - UGA

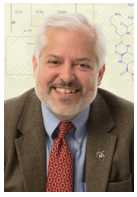
MARCH 2025

In This Issue

2 Recent News & Updates
3-4 Research Highlight
5 Clinical Research Coordinators
6-7 News Around the Lab
8 Community Events
9 Pilot Grants & Seminars
10 Connect with Us

Director's Update

Dr. Nael McCarty



We are happy to provide to you the next edition of the CF@LANTA e-newsletter, and hope that it finds you well. Great things continue to happen in CF@LANTA, across our three missions of research, education, and clinical care. The former two, however, already are suffering from the new attacks on federal funding for the National Institutes of Health (NIH) and the National Science Foundation (NSF). I'm taking this opportunity to educate our community on this issue.

The United States has been home to the greatest biomedical research engine in the world since investments by the federal government blossomed after World War II. This has benefitted all of humanity, both those suffering from diseases like CF and those who are otherwise kept healthy by drugs and diagnostics developed over these years. Keep in mind that most new drugs, including those amazing therapies from Vertex Pharmaceuticals that the CF community knows about, got their start in academic labs typically funded by the federal government. The CF gene was identified in academic labs. It was academic labs that figured out how to study the CFTR protein in low-throughput experiments, generating fundamental knowledge that then allowed pharmaceutical companies to study ways to modulate CFTR protein function in high-throughput experiments. This led to identification of Ivacaftor, Tezacaftor, and Elexacaftor, the three drugs that make up Trikafta. Then, Vertex came back to academic medical centers, like ours, to test those developing therapies in clinical trials with our patients, physicians, and clinical research teams. All of this depends upon the productive relationship between NIH-funded academic labs and investor-funded pharma companies. Indeed, in the absence of federal funding, the development of new therapies – including for the large number of CF patients that don't benefit from Trikafta or the newer Alyftrek – will slow to a crawl.

In a recent article in "The Atlantic," Ian Bogost noted that the current Administration "has frozen, slashed, threatened, and otherwise obstructed the tens of billions of dollars in funding that universities receive from the government" for research and training. Some of this funding literally is needed to keep the lights on in our labs. Did you know that the NIH budget included $85M for CF research this fiscal year? Did you know that, according to data from "United for Medical Research," in fiscal year 2023, the NIH awarded $780M in grants and contracts in the State of Georgia, which supported 11,816 jobs and resulted in $2.18B of economic activity in our state? Clearly, funding for research and training serves the whole nation, but is the basis for moving forward to our shared goals of curing and controlling diseases that impact our loved ones and those we don't even know.

In these trying times, we will be asking our friends to consider whether they can support our programs with philanthropic contributions. No matter your political affiliation, we in the scientific community are going to need your help. If you would like to discuss any of these points with me, please reach out: namccar@emory.edu.

We also want to note the loss of one of our most vocal advocates, Mr. Andy Lipman, who died on January 17, 2025. Andy was a CF Warrior from day one, a loving husband, father, brother, and son, and a source of strong support to all like him who fought this devastating disease. We share in the grief felt by his family and all those across the CF community. May his memory be a blessing.

With best wishes,
Nael

Recent News and Updates

CF Family Education Day

Rachel Linnemann, MD, Director of the Cystic Fibrosis Care Center and Co-Director of the Pediatric Cystic Fibrosis Program at Children's + Emory, and Deepika Polineni, MD, MPH, Keynote Speaker

The Children's Healthcare of Atlanta and Emory University Cystic Fibrosis Care Center held CF Family Education Day on Saturday, February 1, 2025, at the Arthur M. Blank Hospital. Center Director Rachel Linnemann, MD, kicked off the morning, providing an overview and state of the center. Dr. Linnemann noted that the Children's + Emory Care Center cares for a diverse set of 750 people with cystic fibrosis. Our Care Center received the CF Foundation Outstanding Care Center Award at NACFC in September 2024 and continues to focus on quality improvement measures as part of the Cystic Fibrosis Learning Network, currently working on the care model based on the new guidance from the Cystic Fibrosis Foundation. The approval of Alyftrek and expansion of Trikafta to additional genetic profiles provides further treatment options for our patients, and exciting clinical trials are underway at Emory-Children's.

Our keynote speaker, Deepika Polineni, MD, MPH, Professor of Pediatrics, Allergy & Pulmonary Medicine and Director of the Cystic Fibrosis Center at Washington University, St. Louis, spoke on "CF Gene Therapies: Where We Are Going and Where We Are Now." The day concluded with Rapid Fire talks, including content on Mental Health, GI, and Transition Care by Emily Seibert, LPC, Vivek Shenoy, MD, and Brandi Middour-Oxler, DNP. Families had the opportunity to connect with clinicians, researchers, and each other.

Thank you to all of our in-person and online attendees, as well as the Cystic Fibrosis Foundation and Emory -Children's Care Center team members for making this a successful day!

Winter Bipartite

Children's + Emory Care Center's Winter Bipartite, held on December 11, 2024, provided an opportunity for clinical and research staff serving both children and adult patients with cystic fibrosis to share updates and connect with each other on their current and future work. Dr. Matt Hazen provided an overview of the Center and a presentation on prenatal modulator use for fetuses with CF. Dr. Vivek Shenoy spoke about GF gastroenterology, Dr. Cynthia Tsai discussed CF adult care, Eric Hunter provided details on clinical trials for 2025, and Chris Driggers addressed the next generation registry and investigator initiated CF research studies. Dr. Randy Hunt and Dr. Rachel Linnemann presented awards to the following recipients. Congratulations to the awardees who serve our patients so well and contribute to their teams, making a significant impact all around.

Ms. Barbara Henry Hero Award for Adult CF Care: ***Gabby Do, PharmD, BACAP***

Dr. Daniel Caplan Hero Award for Pediatric CF Care: ***Miah Starks, MBA, RRT, CPFT, RHIT***

Dr. Arlene Stecenko Leadership Award for Excellence in CF Care: ***Mariela Duval, PharmD/RPh***

Research Highlight

FDA Approves Alyftrek Treatment in Children with Cystic Fibrosis, from Data Published in High Impact Journal by Children's + Emory Specialists Drs. Rachel Linnemann and Ajay Kasi

CF-AIR and CF@LANTA primary investigators Rachel Linnemann, MD and Ajay Kasi, MD, along with collaborators, recently published impactful journal articles outlining the results of phase 3 clinical trials in people with cystic fibrosis (CF) that led to the U.S. Food and Drug Administration (FDA) approval on December 20, 2024 of the new triple-combination modulator Alyftrek (vanzacaftor/tezacaftor/deutivacaftor).

Alyftrek is a once-daily cystic fibrosis transmembrane conductance regulator (CFTR) modulator for people with cystic fibrosis ages 6 and older who have a mutation that is eligible for Trikafta or one of 31 rare mutations that had not been approved previously for a modulator.

Dr. Kasi was a national principal investigator for the trial for children ages 6-11 years, and second author on the journal article titled, "Vanzacaftor–tezacaftor–deutivacaftor for children aged 6–11 years with cystic fibrosis (RIDGELINE Trial VX21-121-105): an analysis from a single-arm, phase 3 trial," published in *The Lancet Respiratory Medicine* on January 1, 2025.

Dr. Linnemann was a national principal investigator for the trial for children ages 12 and above, and co-author on the journal article titled, "Vanzacaftor–tezacaftor–deutivacaftor versus elexacaftor–tezacaftor–ivacaftor in individuals with cystic fibrosis aged 12 years and older (SKYLINE Trials VX20-121-102 and VX20-121-103): results from two randomized, active-controlled, phase 3 trials," also published in *The Lancet Respiratory Medicine* on January 1, 2025.

The FDA approved Alyftrek based on results from these clinical trials, which compared the drug to Trikafta, along with lab test data that showed additional rare mutations were responsive to Alyftrek. With administration once a day, Alyftrek provides a simpler dosing regimen for people with cystic fibrosis, as compared to twice daily for other modulators. The studies were funded by Vertex Pharmaceuticals. Overall, nine patients with cystic fibrosis enrolled and participated in these studies from the Emory + Children's CF Care Centers.

The approval of vanzacaftor-tezacaftor-deutivacaftor is a significant milestone for people with cystic fibrosis. The multicenter international study showed that vanzacaftor-tezacaftor-deutivacaftor was safe, well-tolerated, and improved lung function comparably to Trikafta, while reducing sweat chloride levels further than Trikafta. In the pediatric study, nearly all participants achieved sweat chloride levels below the diagnostic threshold for CF and more than half had normal levels highlighting the efficacy of this drug. Improvement in CFTR function could prevent progression of cystic fibrosis.

-Dr. Ajay Kasi

Research Highlight

FDA Approves Alyftrek Treatment in Children with Cystic Fibrosis, from Data Published in High Impact Journal by Children's+Emory Specialists Drs. Rachel Linnemann and Ajay Kasi (continued)

Drs. Linnemann and Kasi would like to thank the Cystic Fibrosis research team, particularly the Clinical Research Coordinators, who played a significant role in conducting the study at Emory + Children's CF Care Centers.

These new modulators are life changing therapeutics for many individuals with cystic fibrosis, although there are many unanswered scientific questions remaining, particularly the longer-term effects of increasing CFTR activity in the growing child. In addition, 2025 will bring testing at our Emory + Children's CF clinical trials site of more exciting and potentially life changing treatments for our patients with cystic fibrosis, including the bionic pancreas for those with CF-related diabetes and an mRNA CFTR genetic therapy, which is agnostic to CFTR genotype.

Concurrent with this announcement, the FDA also approved the expansion of Trikafta (elexacaftor/tezacaftor/ivacaftor) to people with cystic fibrosis ages 2 and older who have at least one of 94 rare mutations in the CFTR gene, including the N1303K variant. Emory + Children's researchers Dr. Linnemann and Dr. Eric Sorscher, along with collaborators, recently published an impactful journal article in The Lancet Respiratory Medicine, which outlined the results of a clinical trial in people with cystic fibrosis encoding the N1303K variant that demonstrates significant positive outcomes for Trikafta. The article, titled "Evaluation of elexacaftor–tezacaftor–ivacaftor treatment in individuals with cystic fibrosis and CFTR-N1303K in the USA: a prospective, multicentre, open-label, single-arm trial," can be found here.

The Lancet Respiratory Medicine is a world-leading respiratory medicine and critical care journal with an Impact Factor of 38·7, ranking first among 54 critical care and 100 respiratory system journals globally.

Dr. Linnemann serves as the Director of the Cystic Fibrosis Care Center at Children's Healthcare of Atlanta and Emory University, and she is also an Associate Professor, Department of Pediatrics at the Emory University School of Medicine.

Dr. Kasi is a pediatric pulmonologist at Children's Healthcare of Atlanta and an Associate Professor of Pediatrics at Emory University School of Medicine. He is the Medical Director of the Technology-Dependent Pulmonary Program at Children's Healthcare of Atlanta.

Dr. Sorscher is a Georgia Research Alliance Eminent Scholar, the Hertz Endowed Professor in Cystic Fibrosis Research, and Professor, Department of Pediatrics at Emory University.

> ***It is exciting that Alyftrek will be an alternative treatment option for people with CF, including those who cannot tolerate Trikafta, and it will be available for additional individuals who weren't previously eligible for any modulator. We expect Alyftrek's once daily dosing will also facilitate adherence, since modulators need to be taken with fat-containing food.***
> ***-Dr. Rachel Linnemann***

Spotlight on Clinical Research Coordinators

Emory University, Children's Healthcare of Atlanta, and the Cystic Fibrosis Foundation have made significant investments in CF clinical care and clinical research at the Children's-Emory CF Care Center. Our team of clinical research coordinators is vital to the success of our clinical trials. Clinical Research Coordinators are responsible for a number of tasks, including the recruitment and screening of participants, collecting and managing data, monitoring any adverse events, ensuring regulation compliance, and managing study sites. We would like to highlight each of our CRCs and thank them for their time, energy and devotion to the success of our trials!

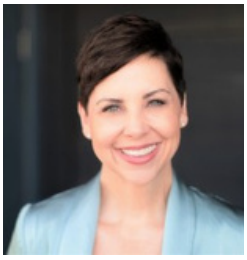
Cynthia Azike
Dinisha Batchelder
Brian Buehler
Katy Clemmer
Desiree Coker
Joy Dangerfield
Chris Driggers
Eric Hunter
Linque Martin
Alysia McKenzie
Demetria Oliver
Lucas Silva
Ashleigh Streby
Jocelyn "Josie" Villalobos-McNeany

Recent Awards & Accomplishments

Kudos to...

Dio Kavalieratos, PhD and Brandi Middour-Oxler, DNP

The Cystic Fibrosis Foundation (CFF) released its consensus guidelines in December 2024 with the article titled "Addressing pain in people living with cystic fibrosis: Cystic fibrosis foundation evidence informed guidelines" published in the *Journal of Cystic Fibrosis*. Drs. Kavalieratos and Middour-Oxler are both authors on this publication, which is a cumulation of three years of work with multidisciplinary experts around the world including people with CF and their loved ones.



Rabindra Tirouvanziam, PhD

Received the *Schinazi Family Distinguished Professorship in Biomedical Research*. He is one of 31 newly endowed professors who were honored during the Celebration of Faculty Eminence for their commitment to advancing scholarship and research while enriching student experience. Dr. Tirouvanziam recently received funding for two grants, “In Vitro Assessment of Chemical Mixture-induced Airway Inflammation in Healthy and Diseased Lungs” and “Evaluation of DDP-1 inhibition on pathological conditioning of neutrophils transmigrated to cystic fibrosis airway fluid supernatant in vitro.”

Nael McCarty, PhD

Received the NOA for his R01 titled “Mechanisms linking CFTR to dysregulated barrier function, insulin receptor function, and glucose transport in the CF lung,” funded through NIH NHLBI, with an award amount of $3,652,470.

Kathryn Oliver, PhD

Received the NOA for her R01 titled “Tuning translation efficiency to overcome refractory defects in CFTR,” funded through the NIH NHLBI, with a total award amount of $3,090,095.

Brian Vickery, MD

Emory-Children’s was one of 10 U.S. sites to conduct the NIH-sponsored Phase 3 OUtMATCH trial of omalizumab in food allergy, which met its primary endpoint and was published in the *New England Journal of Medicine (NEJM)* last February, leading to the concomitant FDA approval of omalizumab for this indication. *National Geographic* named this study one of "7 Medical Breakthroughs That Gave Us Hope in 2024" and one of the NEJM's 14 Notable Articles of 2024. This study is changing practice at Children's and around the country as patients as young as 1 year of age, who previously had no viable treatment options and are now able to be protected from the risks of anaphylaxis.

Recent Awards & Accomplishments

Kudos to...

Ben Kopp, MD for his selection as a 2025 Physician EDGE participant at Children's Healthcare of Atlanta. In addition, he was senior author on two publications in January 2025: "Secondhand vape exposure regulation of CFTR and immune function in cystic fibrosis," in *American Journal of Physiology, Lung Cellular and Molecular Physiology* and "Flow cytometric measurement of CFTR-mediated chloride transport in human neutrophils," in *Journal of Leukocyte Biology.*

Kimmy Dickinson, MD, MPH, on her senior authorship of the recent journal article titled, "It's Like You're Feeding Your Child Twice: Barriers and Facilitators to Human Milk Feeding Children With Cystic Fibrosis," published in *Pediatric Pulmonology* on 27 January 2025.

Kymry Jones, PhD on her 2024 Unsung Hero Award at the Children's-Emory 2024 Annual Faculty, Physician & Staff Awards Ceremony (*pictured with Dr. Nael McCarty, left and Dr. Ben Kopp, right).*

Katie Foye on her 2024 STAR Award at the Children's-Emory 2024 Annual Faculty, Physician & Staff Awards Ceremony.

Cystic Fibrosis Foundation Funds *"CF Exposome"* Project to Study Preventable CF Complications

We are pleased to announce that **Dr. Tanicia Daley Jean-Pierre (L)** and **Dr. Jessica Alvarez (R)** have received funding from the CFF for their new project, ***CF Exposome***. This project aims to find out how environmental factors affect health complications in people with cystic fibrosis.

CF Exposome combines four studies to understand how different body systems interact in CF. Dr. Daley will examine how the environment affects CF-related diabetes and endocrine function, while Dr. Alvarez will study the impact of nutrition, body composition, and metabolism. Dr. Benjamin Kopp will focus on environmental toxins' effects on lung health and immunity, and Dr. Kathryn Oliver will research the role of genetics and CFTR biology in CF complications.

By combining these studies, CF Exposome aims to find ways to prevent complications and improve health outcomes for everyone with CF. We look forward to sharing updates as the project moves forward!

Upcoming Community Events

ShamRockin' For A Cure

Saturday, March 15th
Union Hill Park, Alpharetta

Celebrating St. Patrick's Day, Supporting the Cystic Fibrosis Foundation

ShamRockin' for a Cure is an award-winning Party for a Purpose that has raised over $4 million dollars for the Cystic Fibrosis Foundation. Join us for an evening out in Alpharetta! Explore dishes from over 25 restaurants and a top shelf open bar, enjoy live music by pop artist Justin Borgman, shop our auction, and more. This is a favorite St. Patrick's Day tradition. Click here for more information and to purchase tickets. ShamRockin' for a Cure is a 21+ event.

Atlanta Great Strides

Saturday, May 17th
Suwanee Town Center

Great Strides towards a Cure for Cystic Fibrosis

Join us for a 3-mile walk to support our mission to cure cystic fibrosis. This Cystic Fibrosis Foundation community event brings together those working towards a cure for CF, raising awareness, support and funds. Great Strides is the CFF's largest national fundraising event. Click here for more information and to register.

Pilot Grants & Seminars

LOI 2025 Georgia Cystic Fibrosis Core Center P30 Pilot & Feasibility (P&F) Program

The Georgia Cystic Fibrosis (GACF) Core Center is pleased to announce the Pilot & Feasibility Program Request for Applications (RFA) to support research in cystic fibrosis (CF) disease pathogenesis and/or prevention. We are seeking to fund up to **two pilot projects with $50,000 in total direct costs per year for up to two years**.

Key Deadlines

Letter of Intent (LOI) Due: March 10th by 5:00 PM ET

Selected LOIs will be invited to submit a full proposal

For full details on this RFA and to submit your LOI, please click here to visit the InfoReady page

Upcoming CF-AIR Seminar Series Spring Schedule

CF-AIR Seminar Series Spring 2025 Schedule			
Date	**Time**	**Type**	**Presenter**
6-Mar	1:00-2:00	Guest Lecturer	Peter Jorth, PhD (Cedars Sinai)(*HSRB II N600)
13-Mar	1:00-2:00	Journal Club	Diego Moncada, PhD
20-Mar	1:00-2:00	CF-AIR Research Highlight	Brittany Ross, PhD (Georgia State)
27-Mar	1:00-2:00	CF-AIR Research Highlight	Rabindra Tirouvanziam, PhD & Joshua Chandler, PhD
3-Apr	1:00-2:00	Guest Lecturer	Jarrad Scarlett, MD, PhD (Seattle Children's)
10-Apr	1:00-2:00	Journal Club	Sam Durfey, PhD
17-Apr	1:00-2:00	Trainee Focused	Brian Dobosh, PhD
24-Apr	1:00-2:00	CF-AIR Research Highlight	Julie Champion, PhD
1-May	1:00-2:00	CF-AIR Research Highlight	Arlene Stecenko, MD
8-May	1:00-2:00	Guest Lecturer	Charles (Chuck) Esther, MD, PhD (UNC-Chapel Hill)
15-May	1:00-2:00	Trainee Focused	Courtney Fernandez, PhD (Dalton State)
22-May	1:00-2:00	CF-AIR Research Highlight	Lokesh Guglani, MD
29-May	1:00-2:00	Social	Birthdays

Please contact Megan Stewart at megan.stewart@emory.edu for questions or additional information on our Seminar Series

Around the Research Organization

Click for more information and to register

Pediatric Core Services

Offer the equipment, tools, and skilled professionals to make research faster and more impactful. From imaging and data analysis to clinical trial support, our pediatric cores provide key resources and expert support to help researchers at every step.

Researchers, check out details about our pediatric research core services here or via the QR code:

For any questions or details, please reach out to Cores Administrator, Julie Flores.

Connect with CF-AIR & CF@LANTA

Visit Our Websites

CF@LANTA

CF-AIR

Join Our Listserv

Would you like to receive information and calendar invites about CF-AIR & CF@LANTA Seminars and other news? Email megan.stewart@emory.edu and request to be added to our listserv!

CF-AIR is now on BlueSky! Follow us and tag us @emory-choa-cfair.bsky.social

Follow and tag us on X @EmoryCHOA_CFAIR and @CFAtlanta