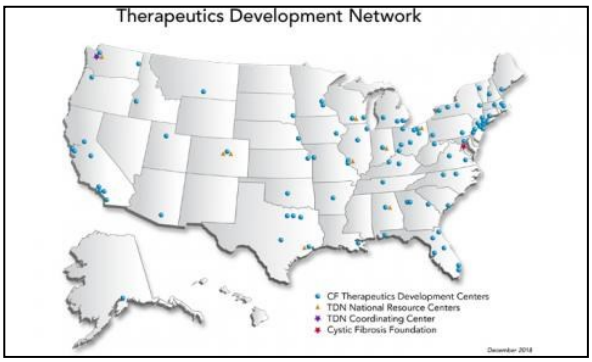
CF FAMILY NEWSLETTER

Spring 2023

UNC HEALTH Children's

A Regional Cooperative

The success of CFTR modulator therapies has led to fewer people with CF eligible or available for participation in clinical trials, while the number of new therapeutics in the full CF Therapeutics Pipeline (https://apps.cff.org/trials/pipeline) has remained constant, and in some areas like genetic therapies has actually expanded. The Cystic Fibrosis Foundation and the CF Therapeutics Development Network (TDN) leadership worked together to address this imbalance.



One idea was to help all CF Centers within regions of the country make referrals easier for people who were interested in participating in clinical trials not available at their home CF Center. In December, an inaugural national meeting (by Zoom, of course) for all CF Center leaders and TDN research programs rolled out this exciting initiative. Our program at UNC is in the Carolinas region, which includes the active CF TDN teams at Wake Forest Baptist, MUSC and USC in South Carolina together with the other CF Centers at Duke, Levine Children's/Atrium Health and the affiliate CF programs in Greenville and Asheville.

We are meeting regularly with the groups in our region to know everyone better and understand the most efficient ways to contact key people and get regular updates on the planned and ongoing research studies at each CF Center. We want any patient we refer to another program (or who may be referred to us at UNC) to have full confidence that their experience will be outstanding and fulfill their wish to participate in a CF clinical trial successfully.

We are one of 20 cooperatives across the country. To make this work smoothly the TDN provides each team a set of tools to streamline referrals and maintain a tight connection between the CF research team and the CF care team, since once a referral is made the medical care will be maintained by the home CF team, and communication around any issues must always be a two-way street.

The hope is that, despite new challenges, we will be able to continue testing the potential for new therapies, whether designed to treat infection, moderate inflammation or enhance clearance of lung secretions, or evaluate the new generation of mRNA and genetic therapies to restore normal CFTR function for those who do not have a CFTR modulator option. Our success with new therapies so far inspires us to expand breakthrough therapies to everyone with CF.

Submitted by George Retsch-Bogart, MD

Inside this Issue:

Trikafta for 2-5 year olds—It's HERE!

Currently Trikafta® (elexacaftor/tezacaftor/ivacaftor), the triple combination CFTR modulator is approved for people with CF 6 years and older with at least one copy of the F508del mutation or one other qualifying mutation. We have all been eagerly awaiting the FDA's approval for Trikafta® for children aged 2-5 years of age. While it has not been approved yet as of the writing of this article, the FDA is expected to make a decision about approval by April 28, 2023, at the latest.

The results of the clinical trial studying Trikafta® in children 2-5 years old were presented at the North American Cystic Fibrosis Conference in November 2022 and the study will be published soon. This study was primarily to determine the appropriate dose for children in this age range and the safety of the medication. In this study, 75 children who had at least one copy of the F508del mutation received Trikafta® for 24 weeks, in addition to their usual CF treatments and medications. Trikafta® was found to be safe and effective in this age range, with average sweat chloride decreasing by 57.9 mmol/L and improvements in fecal elastase values. Common adverse events included cough, elevated liver enzymes, and rash, which were similar to prior studies in older age groups.

As we gear up for the expected FDA approval for Trikafta® in children 2-5 years of age, your CF team has a list of everyone who will be eligible. Here's what to expect once it is approved:

- Your child should have a visit with the CF team (either in-person or virtual) if it has been more than 3 months since his/her last visit.
- Obtain liver function tests (blood test) within 3-6 months before starting Trikafta®.
- Obtain an eye exam to screen for cataracts, ideally before starting Trikafta®.
- Have an education session with one of the clinic CF Pharmacists (either in-person or virtual) to review important information regarding the medication and to complete some paperwork.
- Sign and return the Vertex GPS enrollment form. Your CF Pharmacist will also discuss some of the nuances regarding the new copay assistance program with you during your visit.

Once all the above steps are completed, a prescription will be sent to the specialty pharmacy and your CF team will work with your insurance company for approval. When the medication is approved, the specialty pharmacy will contact you to set up shipment. This process may take a few weeks, depending on your insurance coverage.

Routine monitoring while on Trikafta® includes liver function tests every 3 months during the first year, as well as yearly eye exams to screen for cataracts until your child turns 18 years old. Stay tuned for more information about the mobile eye exam bus coming to a location near you! If you have any questions, please feel free to reach out to your CF team.

Submitted by Charissa Kam, PharmD.

Join in on CF Parent Education Nights!

Don't miss an opportunity to join upcoming virtual workshops to help you navigate life as a parent of a child with Cystic Fibrosis! You can find out more, watch previous workshops, and sign up here:

www.cfparenteducation.com

Keeping Your Bones Healthy

People with Cystic Fibrosis (CF) can be at a higher risk for bone disease. The Cystic Fibrosis Foundation recommends that all people with CF be evaluated for bone health. Your CF team is working together on screening and preventing bone disease. Starting at age 8, we will be screening patients for certain risk factors. These risk factors can include low vitamin D levels, low body mass index (BMI) and certain types of medications that can lead to lower bone mineral density (e.g. long term use of oral corticosteroids like prednisone). If your CF team identifies a risk factor, you may be referred for a dual-energy X-ray absorptiometry (DEXA) scan. This is a painless procedure that uses X-ray to check bone mass or bone density. Your CF team will follow-up on the results and make a plan on how to best keep your bones healthy. This will include a review with your CF dietitian of your nutrient intake of calcium, vitamin D and vitamin K. Feel free to ask your CF team any questions about this new process.

Submitted by Kelly Baumberger, RD, LDN, CSP

CF R.I.S.E. Returns

Responsibility. Independence. Self-care. Education.

In an effort to help better prepare you for your eventual transition to adulthood, we are starting to implement the CF R.I.S.E. program back into the clinic. Some of you might remember completing some of the modules prior to the pandemic. For many it will likely be new!

CF R.I.S.E. is a program that covers various topics related to CF care and health from airway clearance to emotional wellness to school/work and CF-related Diabetes. With such a range of topics, the goal is to help parents and family members gradually transition responsibility for treatments, medications, and care from the adults to the child/adolescent. The CF RISE program has modules for individuals as young as 10, and spans all the way up to 25 years old.

We are going to start slowly and begin with patients who are closer to the time of transitioning, those in the 16+ age range. If you are in this age group, you might be asked in clinic to fill out a module, either on paper or on the computer to test your knowledge (it's important to remember this is not actually a test, it just lets us know what we need to keep teaching you!)

For those of you under 18 years old, your parents or guardians will need to give permission for you to participate, and we will get official consent in clinic, either electronically or on paper.

We are excited to begin to have CF R.I.S.E. be a part of your care again and help prepare you in the best possible way for adulthood! If you are interested in looking at the website and learning more check out https://www.cfrise.com/ or ask your nurse or social worker for more information.

> *"...It is important to understand why you do certain things. You are more likely to do things if you understand why." -CF R.I.S.E. User*

Submitted by Ellen Penta, LCSW.

New to UNC: Meet Our New CF Team Members!

Andre Espaillat, MD, MPH

I did my residency at Texas Children's Hospital: Houston.

My initial interest in pulmonology stems from my personal history with Asthma. However, I started residency on the pulmonology team, and we had over 15+ patients with Cystic Fibrosis. I loved getting to know the kids and families while also treating their multi-organ disease. Those connections were like nothing else I experienced during the rest of my residency and knew it was something I wanted in my future career.

My wife and two goldendoodles, Bella and Maggie, are the most important people in my life. I'm a sport fanatic, and most importantly, I'm a HUGE Florida Gator fan.

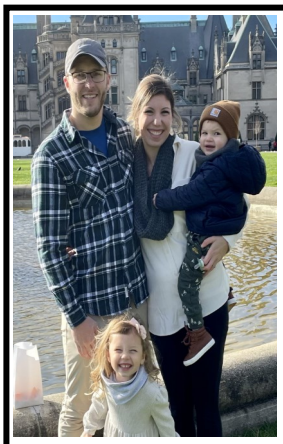
I look forward to meeting you when you come to clinic!

Cindy Williams, MD

I did my residency (plus a chief year) at Arnold Palmer Hospital for Children in Orlando, FL.

Cystic Fibrosis was my first true introduction to pediatric pulmonology during a medical school rotation with CF clinic on Day 1 of the rotation. I loved how everyone from the dietitian to the social worker to the physician (among others) came together to offer the best care to each patient. Fast forward to halfway through my pediatric residency, and Trikafta became approved. I got to see how it affected so many people with CF, and I was hooked after that!

My free time now is mainly spent spending time with my husband and running after my daughter, who turned 1 in April. The three of us are always looking for new ways to have fun and explore the area!



Emily Shill, BSN, RN, CPN, CCRN

I have been an adult and pediatric critical care nurse at the bedside for 9 years, and worked at the UNC Children's Specialty clinic for 2 years through the pandemic. I spent 2022 travel nursing to ICUs and made my way back to pediatric Pulmonology as soon as I heard they were hiring! I had worked with the Pediatric Pulmonary team during my time at the Raleigh Clinic and really grew to love working with their team and their patients.

I am SO excited to join this fantastic team! The Pediatric Pulmonary team work so well together, everyone is so supportive, compassionate, and hard-working and I was very motivated to join such a great group of people. I also really love working with pulmonary patients because the lungs are so fascinating to me! On top of that I love getting to create deep connections with my patients and their families, so taking care of kids with chronic illnesses means I get to spend a lot of time with them throughout their medical journey!

I am married to my High school sweetheart who is also my very best friend in life and we have two beautiful and busy children: Our daughter Olivia is 4 and our son Benjamin is 2. We also have a dog named Thor! We spend a lot of time outside walking, having bonfires, and spending time with family and friends. We also enjoy traveling!

New to UNC: Meet Our New CF Team Members! (cont).

Grace Morningstar, MPH

I just graduated from the MPH program at Gillings School of Public Health here at UNC. Before that, I worked as a clinical associate at Duke for about a year.

I love working with kids, and CF is a disease that I've had an interest in since a family friend was diagnosed when we were (a lot) younger. I'm most excited about being able to contribute to the clinical studies Will, Cameron, and Marianne are doing, and to hopefully make a positive impact on the quality of life of CF patients through the innovations being introduced.

I'm a big runner. I used to be on the UNC Marathon Team in college, and I'm thinking about getting back into training to participate in some half marathons this upcoming year! I also have a dog, Albie, who is the light of my life; he enjoys chewing hard plastic and eating full rotisserie chickens while I'm not looking.

I just want to thank everyone for being so welcoming thus far, and let them know that I'm excited to be here!

Rosie Hernandez Lainez

I am a California native but have lived in North Carolina since 2016 when I decided to go to Medical Assistant school. I worked in General Pediatrics fresh out of school. I did my pediatric rotation at this practice and was nervous as I considered myself someone that "would never work with children" but that significantly changed when I started this rotation, and luckily enough they offered me a position, so I stayed after graduating.

While working in general pediatrics, there were so many families that left imprints on me and one was a pair of siblings with CF. A year later I started working at UNC Adult Pulmonary Clinic and then research caught my attention. The CF research team was hiring a new coordinator, so it felt very fitting, and I'm very grateful to be here. I am excited to get to know CF families and build trust with them.

In my free time, I love spending time outdoors, I rescued a pup 3 years ago, so we like to go on hikes together. I am in general, an enthusiast, anything that catches my attention I like to learn all about.

A Fond Farewell

Many of you know that Jennifer Leandro left our division in January to take a new position closer to home in Wilmington NC. She has joined a group focused on maternal fetal health care, as a Clinical Supervisor at Novant Health Maternal Fetal Medicine program. Jennifer joined our division in the fall of 2013 after working for years as a staff nurse on the inpatient surgical unit of UNC Children's. She brought her experience, enthusiasm and passion for children's health to her new position and sustained it throughout the decade she was with us. During those years, she made strong connections to the NC Cystic Fibrosis Chapters and to the national Cystic Fibrosis Foundation programs. She participated in the CFF Volunteer Leadership Conference, served on the CFF Education Committee and more recently completed an intensive course in leadership sponsored by CFF at The Institute for Excellence in Health and Social Systems at The University of New Hampshire. She was also a key member of our CF Center's Quality Improvement team.

In addition to her love of nursing, whether as a clinic nurse or a camp nurse during summer weeks, she valued the close relationships she developed with our families. She always looked forward to the latest updates about school projects, family vacations, graduations, concert performances and other events affecting our families. As many of you know, she was always ready to jump in and help solve new issues or problems, especially knowing how to track down the physicians or other team members to help with the solution.

We all miss her and her devotion to our CF Program and wish her well in her new role with that very lucky practice in Wilmington.

Submitted by George Retsch-Bogart, MD.

RESEARCH

Many CF studies are ongoing at UNC! More information on clinical trials being done here at UNC and around the country can be found on the Clinical Trials page at www.cff.org or by searching www.clinicaltrials.gov. You can also contact one of your hard-working Pediatric CF Research Coordinators directly:

Rosie Lainez at Rhlainez@email.unc.edu OR Thomas Shields at shieldt@email.unc.edu.

The CF Hear Project

Our CF team is leading a project, along with several other centers across the country, to help screen our patients with Cystic Fibrosis for hearing changes. Certain antibiotics called aminoglycosides (which can be given either by an IV or inhaled) are commonly given to people with CF for the treatment of bacterial infections such as Pseudomonas. Tobramycin and amikacin (or Arikayce®) are examples of aminoglycosides. Exposure to these antibiotics, as well as some other medications such as azithromycin, has been shown to cause hearing loss in some patients. For this reason, hearing loss screening is recommended for CF patients who use these medications.

Unfortunately, there is currently no standard hearing loss screening for the many CF patients who are treated with these medications. The CF HEAR project at UNC aims to create and spread a quick and effective hearing screening procedure for CF patients who are at risk of hearing loss.

At the beginning or end of standard clinic visits, patients who have been exposed to these medications will participate in a hearing screening that uses a tablet and headphones to test their hearing. Screenings should only take approximately 5-10 minutes, and if hearing changes are detected, we will connect patients with audiologists or ENT physicians for further evaluation. Your care team will also discuss whether any changes in your treatment plan should be made.

As we begin this screening, we greatly appreciate your thoughts and suggestions on the hearing screening process, and what changes could improve the experience. You may be asked to complete a voluntary survey about the process. Your feedback will not only help other people with CF at UNC, but at CF centers all over the United States!

Submitted by Cameron McKinzie, PharmD & Grace Morningstar, MPH.

If you attend year-round school or have any summer camps coming up that require medical forms , please reach out to your CF nurse as soon as possible so these can be completed in time.

Help Us Make the Newsletter Better!

If you would like to submit an article or have an idea for the next CF Family Newsletter, please contact Kelly Moormann at kelly.moormann@unc.edu.

Contact Us

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984-974-1000

Visit us on the web at:
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UNC MyChart: https://myuncchart.org

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