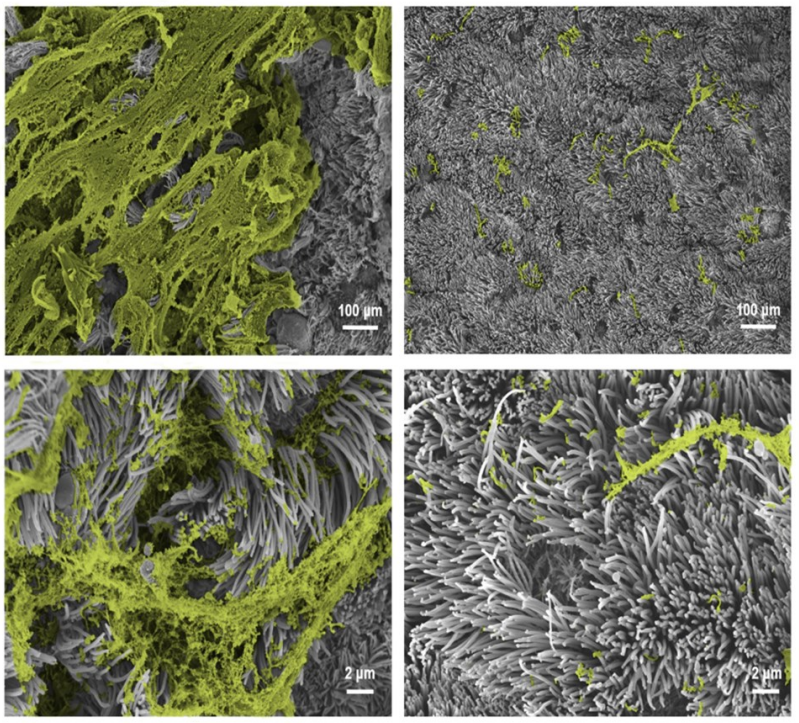
CF FAMILY NEWSLETTER

Fall 2021

UNC HEALTH Children's

Microscopic Mucus

Many people with CF are benefitting from the recent medical breakthrough of CFTR modulators. These small molecules, taken in the form of daily tablets, restore CFTR function, which has shown to significantly improve lung function, increase body weight, and reduce the rate of hospitalizations. Ivacaftor (Kalydeco™), the first approved CFTR modulator, is highly effective, but because it only worked on "mild" mutations (e.g., G551D), this drug could only be prescribed to a small fraction (5-7%) of the CF population. However, in the fall of 2019, just before the COVID-19 pandemic hit the USA, Trikafta™ was approved as a highly effective therapy against the most common CFTR mutation, F508del, which affects more than 80% of people with CF. Trikafta™ is a game changer for many people, but the actual mechanism by which CFTR modulators reversed CF mucus abnormalities remained unclear.



During the past decade, researchers determined that there were many factors contributing to the characteristic properties of the thick, sticky CF mucus. Our lab at the UNC Marsico Lung Institute recently published a study in the European Respiratory Journal investigating the dominant change that occurs in CF mucus after treatment with CFTR modulators. In this study performed in cell cultures, we used bronchial cells from CF donors carrying the G551D or the F508del mutations and treated the CF cells with ivacaftor or Trikafta™, respectively. This study showed that CFTR modulator treatment resulted in increased mucus transport, decreased mucus viscosity (thickness and stickiness), and a looser mucus architecture, which facilitated the removal of airway mucus from the cell surfaces.

The images here were taken using scanning electron microscopy, a special technique that provides detailed images of the surfaces of cells. They show an aerial or top view of CF bronchial cells before and after Trikafta™ treatment at low and high magnification. Before treatment, mucus, which can be seen in green, covers the airway cells in thick sheets. However, after treatment, the cell surfaces have very little mucus sticking to them. This allows you to see cells with hair-like projections called cilia that help transport mucus out of the airways and demonstrates the profound effect of this drug. We have also found that prolonged hydration of mucus is the dominant change produced by CFTR modulators and is able to reverse the problematic properties of CF mucus. With the recent FDA approval of Trikafta™ for individuals 6-11 years old, the future of cystic fibrosis treatment is looking bright.

For more information about research in the Ehre Lab, check out: https://www.med.unc.edu/marsicolunginstitute/directory/camille-ehre-phd/

Education and Activity Scholarships for CF Patients

There are so many great opportunities for Cystic Fibrosis patients to apply for education scholarships as well as scholarships for other fun activities like surfing, camp, and (free) custom birthday cakes!

Scholarships are available for community college, online classes, trade school, four-year colleges and even a few for graduate programs. There are even scholarships available for family members! Some offer a few thousand dollars up to tens of thousands of dollars. Each have their own criteria, often they include an essay, an application, and sometimes a letter from your CF center. Some require a certain GPA or community involvement; regardless of your qualifications, it is always worth looking and applying. There are scholarships that are through smaller local organizations and others that go through larger national organizations. Some examples are: AbbVie offers scholarships up to $23,000, you can find out more information at https://www.abbviecfscholarship.com. The Bonnell Foundation (https://thebonnellfoundation.org/) offers scholarships as well as financial assistance to families in need. Dylan's Dream, an organization honoring a young man who passed away, offers scholarships in the amount of $2500, information can be found at https://www.dylansdream.org/apply-for-scholarship/.

If educational scholarships are not on your mind just yet, there are some fun opportunities for kids and families. Just to name a few, a $10 lifetime pass to all national parks, camp scholarships, financial assistance with exercise activities, and custom birthday cakes! Get your custom birthday cake from Icing Smiles at www.icingsmiles.org, your national park pass at https://store.usgs.gov/s3fs-public/access_pass_application.pdf and your free running shoes from www.LetsrockCF.org/kicksback. You can even spend a day learning to surf with the Mauli Ola Foundation, learn more at https://mauliola.org/home. All people with CF are eligible for these opportunities.

This list of scholarships and opportunities, just scratches the surface. Please reach out to your CF Social worker for a complete list; we are here to answer your questions!

Article submitted by Ellen Penta, LCSW

Introducing Tonya Stafford, New Nurse Coordinator

1. Why did you take this job? I have worked with pediatric patients my entire nursing career, 25 years plus. I recently left the bedside 2 years ago to be a nurse educator in that role, and I missed direct patient and family interaction. I am thrilled to be working with such a great team and such wonderful patients and families. I am four weeks in, and I could not be more assured that I have made the appropriate career move.
2. What were you doing prior to this job/ where was your prior experience? I am a pediatric nurse to the core. I started at Children's of Pittsburgh working with the liver and small bowel transplant patients. I worked on 7 Children's from 2001 to 2019 working with a variety of pediatric patients with surgical and medical needs. Prior to this, I was a nurse educator with the Pediatric Trauma Program for two years.
3. What are you most excited about? I am so excited to get to know the Pulmonary population, learn more about each of the specific aspects of the population (e.g., CF, Asthma, PCD, airway complications) and overall thrilled to provide education and be the bridge to the providers. The Pulmonary team has a great reputation in the Children's Hospital, and I am thrilled to be a part of it!
4. Can you share a little bit about yourself? I am a proud Mom of four beautiful children; three girls and a boy. My husband and I are both from Pittsburgh but chose to raise our family in NC since 1999. I am a proud UNC employee and truly believe in the Carolina Care Way. I overall love being outside jogging, hiking, and my most favorite is my time at the beach.

32 Years in the Fight

Andrew Hauser is 32 years old. He holds a master's degree in Mathematical Finance and works as a Quantitative Analyst at Ally Financial. He has been married to the love of his life for almost three years and loves to travel and be outdoors hiking, snow skiing or just experiencing nature. Oh yeah, Andrew also has Cystic Fibrosis and his mutations make him ineligible for the currently approved modulator treatments on the market.

Andrew was born in a small town in Wyoming and while his sweat test was positive for CF shortly after he was born, the doctors thought it had to be a false positive because CF was so rare. Six months later, he was diagnosed with failure to thrive, transferred to a larger hospital and officially diagnosed with CF. He was started on enzymes and other necessary medications and did well for a time. Then he hit adolescence and rebellion began. As time went on, his rebellion turned to a heavy guilt as he felt his family should not have to choose between the amount of food they bought and paying for his medications.

Once in graduate school, he was able to transfer to the Denver clinic and connected with a social worker there. She was able to resource him with co-pay cards that helped pay for his medications. Removing the financial hurdle allowed him to get back on track with all his treatments and live his life looking and feeling more "normal". Removing the financial hurdle and seeing gains in his health helped cement his adherence with prescribed treatments.*

Now as an adult, Andrew's advice for those with CF is to try to enjoy life! He says that while doing some of his favorite things, like travelling, can be a hassle, feeling tethered to medical devices and medications, it only gets easier the more he does it and the more open he is about having CF. New medical devices, like the portable Monarch vest, have made things so much easier for him too. At the end of the day, he simply refuses to let his diagnosis bring him down or stop him and uses his openness about CF, especially while travelling, as an opportunity to teach someone something new that day.

When asked about his accomplishments, he says, " I am most proud of having a full time job. As a kid with CF, it felt like there was always a gloomy cloud over my head, but my parents wouldn't let me sit in that. They pushed me to succeed, and now I have a full and fulfilling life. Also, my marriage! Having a partner with you at all times is so rewarding. She holds me accountable to my treatments too, which is much better than when my parents were telling me to do them!"

Andrew adds that he finds it pretty intriguing that he was born in 1989, when the CFTR was first discovered. He can see clearly (and first hand) the incredible strides that have be taken in CF treatments over the past 32 years. "I don't see it as the disease it used to be. It can be managed, look where we are now! Just think about where we are going, even if it isn't Trikafta, there are so many other treatments coming. Even to see that Trikafta is possible is so cool and like a breath of fresh air, even though I am not eligible. It gives me hope to see the bigger picture and I am excited about our future. I look at [this journey with CF] not as a fear, but as a hurdle to overcome over and over. I feel blessed that I have CF, because it has made me who I am today. I don't know where I would be if I didn't have it. I wouldn't trade anything for where I am today."

**Please reach out to our CF Social Work team at UNC if you have significant financial burdens that interfere with your ability or desire to follow your prescribed treatment regimen. They can help!*

Cystic Fibrosis Multivitamin Update

Aquadek has announced they have discontinued all vitamins. There are a lot of alternatives to Aquadek that you can change to. The brands currently on the market are:

- Complete Formulation MVW http://mvwnutritionals.com/
- DEKAs plus and DEKAs essential https://callionpharma.com/
- Genadek http://mvwnutritionals.com/
- Gamadek http://gamadeks.com/

Exciting news! MVW nutritionals has announced they will have a CF specific gummy available soon. It is Mango flavor. Below is the product information.

mvw
ADEK
GUMMIES™
THE ESSENTIALS
plus Zn

MVW ADEK Gummies are specifically formulated to meet the dietary needs of individuals who have difficulty absorbing fat and fat soluble vitamins, as seen in cystic fibrosis.

MVW ADEK Gummies provide the essential fat-soluble vitamins A, D , E , and K in a water miscible formulation plus zinc. This product also contains the antioxidants beta-carotene, alpha-tocopherol, and zinc. The source of the vitamin D3 is "non-animal." The product is vegan.

MVW ADEK Gummies were designed to meet or exceed the daily fat soluble vitamin recommendations outlined in the Cystic Fibrosis Foundation Nutrition Consensus Statement published in 2002; the recommendations for vitamin D as described in the 2011 Endocrine Society Clinical Practice Guidelines; and Vitamin K as recommended by the 2002 European CF Society Nutrition Consensus Report.

DOSAGE AND ADMINISTRATION

Age 4 to 8 years: 2 gummies daily

Age 9 years and older: 2 gummies twice daily, or as directed by physician. Best taken with meals and supplemental pancreatic enzymes.

Brands vary slightly in content, but all can meet your CF needs when taken at the correct dose. Please discuss recommended dosing with you CF dietitian before changing your vitamin .

The cost of these specialized vitamins can be significant and they are rarely covered by insurance plans. Check out the resources listed below that might help you obtain these vitamins for free. Ask your CF dietitian or other CF team member for help if needed.

- Creon's CareForward Program https://www.creon.com/cfcareforward
- Zenpep's Live2Thrive Program https://www.live2thrive.org/
- Healthwell Foundation Grant for CF https://www.healthwellfoundation.org/

Contact your CF Dietitians at UNC:

Kimberly Stephenson, RD 919-984-3356
KimberlyEri.Stephenson@unchealth.unc.edu

Kelly Baumberger, RD 919-984-3356
Kelly.Baumberger@unchealth.unc.edu

Photo credit: gamadeks.com

RESEARCH

While COVID-19 has effected how we do research at UNC, it has not stopped it! 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:

Ashley Synger at ashley_synger@med.unc.edu OR Fiona Cunningham at Fiona_cunningham@med.unc.edu.

PROMISE

What does the PROMISE study promise to do?

Each time a new CFTR modulator drug (like Kalydeco, Orkambi or Trikafta) was approved, the excitement about its effects to improve lung function or reduce the frequency of exacerbations (which could mean hospitalizations and IV antibiotic therapy) was obvious, but we also wondered about other benefits these remarkable drugs could have. Anticipating these questions, the CF Foundation has supported various studies to gather information about how other organ systems important in CF would respond and how broad the benefits would be. These included the GOAL study for Kalydeco, PROSPECT for Orkambi, and most recently PROMISE for Trikafta. Because of this work we are learning much more about things like what happens to Pseudomonas in people who've had it in their lungs for years, but what about growth and nutrition, control of blood glucose, or improvements in pancreatic function?

With the June 9th approval of Trikafta for children 6 -11 years of age, we began UNC's participation in the pediatric arm of the PROMISE study. As was true for all of these studies, it was essential to have the first visit before Trikafta was started so we could gather information and do a range of tests to establish a baseline or starting value. This would be the point of comparison for all future measurements after Trikafta continued which will be repeated at 1, 3, 6, 12 and 24 months later. To date, 11 children have enrolled in the PROMISE study, with this study being many of the families' first time being involved in research .

The measurements that will be tracked include: symptoms (respiratory and GI tract related), growth (height and weight), lung function (standard PFTs and a more sensitive test called MBW/LCI—which measures how evenly air fills and empties within the lung), throat swab and sputum cultures, lung inflammation and changes in bronchial mucus, measures of liver and pancreatic function, blood sugar regulation, sweat chloride testing, and additional blood samples stored for later testing (as new, more sophisticated tests are developed by CF researchers in the future).

The CF Foundation plans to look at the next youngest group that will be eligible to begin triple combination Trikafta in the future after its approval. It is aptly named the BEGIN study.

We have been so impressed by our young patients and their families who've signed up for this additional work, and are eagerly doing the extra testing beyond their regular clinic visits. Their contributions will help us understand the broader impact of this new, very potent modulator therapy in a younger age group.

Submitted by

George Retsch-Bogart, MD & Ashley Synger, MA

Gratitude on the Journey

Thanksgiving is right around the corner, but we do not wait for this time of year to feel grateful; we feel it every day. One of the blessings for which we are grateful is being at UNC, and for our son's amazing CF Care Team. Our son was diagnosed with CF at 16 days old, and when he was 3 years old, we made the decision to change providers and joined the UNC CF Team. Our son cultured Pseudomonas for the first time only nine months after coming to UNC. Pseudomonas was an intimidating word for us to hear, and it was incredible the way his CF doctor was supportive and devoted to both our son and us as we navigated this new part of the CF journey. After ten months of extra treatments and medicines, our son beat Pseudomonas, and we had tears of joy!

There have been many tears shed in our family during these last five years, but they have not all been from sadness or fear. There have also been tears of joy and gratitude. Tears when I tell my son he needs to do his 28-day extra treatment again and he responds, “Good, I get more reading time with you!”. Tears when I see my daughter curled up on the couch with her brother, shoulder to shoulder, reading him a story during his treatments. Tears when our friends tell us that they have started a business and will be giving part of the proceeds to the CF Foundation. Tears when our son performed like a rock star on his first lung function test. Tears when we receive an e-mail from our son’s doctor on a Saturday morning, answering our questions and giving us the most amazing support and encouragement. Tears when others share tears with us, and show their love and support through their words, encouragement, and actions. We are grateful for these tears and experiences of love and connection.

Every parent reacts differently to having a child with CF, and it took us some time to get comfortable with being more involved in the CF world. We did not participate in Great Strides for the first two years or jump onto various groups and committees, but we are now in a place that we want to reach out and connect more with others in the CF community. We now love participating in Great Strides (Rides). Joining the Family Advisory Board (PFAB) here at UNC has also been an important part of us becoming active in the CF community. Not only do we get to know more parents of children with CF, but we also get to make a difference. As Zig Ziglar said, “change 'got to' into 'get to'.”

Sometimes it can feel uncomfortable to take the first step; please know that if you are interested in making connections with other parents or looking to help make some changes, you can join us on our monthly PFAB meetings. We are always brainstorming ideas for how to improve care for people with CF. It is ok to come and just listen, or to share your thoughts and ideas. All are welcome, and together we can keep making a difference in our precious children’s lives who we love and support every day!

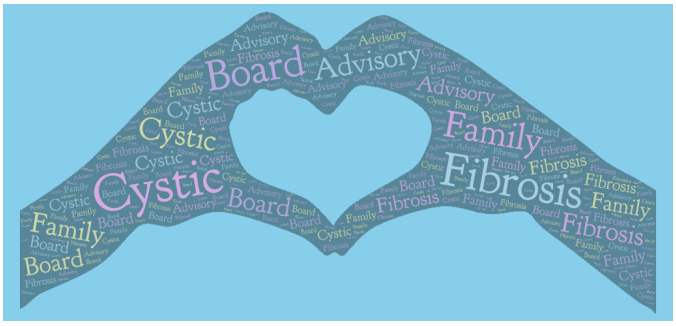
I hope you will join us in feeling an abundance of gratitude this year, not just at Thanksgiving time, but all year round for UNC, all the people who care for our loved ones, and for our loved ones with CF who mean the world to us! It takes a village to care for CF and we are grateful to be a part of UNC!

We would like to share our wishes for our son and all people with CF:

(Continued on page 6)

Gratitude on the Journey (continued)

to always be admired for his strength, instead of pitied.
to rise when he falls and have the resiliency to keep going in spite of the challenges he may face.
to be committed to his health and not just do what is easy.
to always find gratitude instead of the “poor me” mentality.
to always have the courage to be a fighter and never slip into being a victim.
to enjoy every moment he is given, instead of spending his time in anger, fear, or worry.
to understand that sometimes life isn't good or bad, it just is, and he will make the most of what he has.
to always have the wisdom to bring himself back to the roses when he is caught in the thorns.
to believe in himself, instead of doubting. to understand that he cannot control everything that happens, but he can control how he responds to it.
to have a supportive community for his whole life, and not think he must bear this burden alone.
- Two CF Parents



A New Patient/Family Welcome

Who we are: The Pediatric PFAB is a group of mothers and fathers of children with Cystic Fibrosis. We are an active community, with a common bond, and a common goal of doing everything in our power to work with the UNC care team to improve the quality of our children’s lives. We promote clinical research; we work to improve quality of care, support, and safety; we focus on patient-centered care; we are advocates. Advocates for our children, and advocates for you, even if this is the first day you have ever heard the term Cystic Fibrosis.

How we can help: You are not alone. Whether your child is two weeks old or two decades old, diagnosis day is not an easy day. Regardless of your emotions, please know that you have a strong community around you. In your own time, we offer direct conversations with someone who has walked this path before you. A mother, father, sibling, or patient with CF will happily discuss anything you wish to discuss. Ask questions. Ask for feedback. Vent. We are here for you.

Sincerely,
The Members of the UNC Pediatric PFAB

Tanya has been active in trying to support the Cystic Fibrosis Foundation since losing her sister, Sonya, to CF in 2001. Originally from California and a resident of NC since 2003, Tanya is a university professor and mom of 3. When Covid-19 disrupted her regular hobbies and activities, she found herself looking for a new creative outlet. Through a series of happy coincidences, she discovered a passion for handcrafting soap and candles. Before long, she had a substantial supply of soap and candles at home and started giving them away to friends and family. Many of these friends asked if they could buy more products as birthday presents, Mother's Day presents, hostess gifts, and end-of-year presents to teachers. Tanya didn't feel comfortable charging her friends for her products, so her husband suggested that she start a soap and candle business and donate 100% of the profits to the Cystic Fibrosis Foundation. Tanya loved the idea and the business was born! Tanya contributes self-care giftboxes to local families in CF centers in the hope that they can find an occasional moment of peace and relaxation. She also sells products to anyone who loves handcrafted soap and candles -- especially those who want to help the CF community while enjoying (and gifting) top-of-the-line luxury products. You can see Tanya's soaps and candle creations at TLCSoapandCandle.com.

Contact Us

Scheduling: 984-974-1401
(En Español: 919-966-6669)

Pulmonary Office:
919-966-1055 (8am-4:30pm)

CF Nurses:
919-966-1055 (9am-4pm)

Hospital Operator:
984-974-1000

Visit us on the web at:
www.uncchildrens.org/uncmc/unc-childrens/care-treatment/pulmonary-care/cf

UNC MyChart: https://myuncchart.org

uncpedscfcenter

Help Us Make the Newsletter Even Better!

Each newsletter is made up of contributions and feedback from several members of your care teams as well as from patients like you. 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.

Thank you to all those who have contributed to this and past newsletters!