

CF FAMILY NEWSLETTER

Winter 2025



nacfc 2025

The NACFC Edition

This year's North American Cystic Fibrosis Conference was held in October in Seattle, Washington. The conference is always a great place to get the latest on what's what in CF Research and Care. We've highlighted some takeaways in this newsletter, but you can also watch several recordings from the conference on the Cystic Fibrosis Foundation's YouTube channel!

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Blood Lipid Screening

The recent 2025 NACFC Plenary session titled "A Breath of Fresh Age: Redefining Growing Older With Cystic Fibrosis," underscored the growing importance of cardiovascular health for those with CF. The welcome improvements in nutrition and weight resulting from highly effective modulator therapies (HEMT) may also lead to changes in blood lipid levels, such as total cholesterol, LDL-C (low-density lipoprotein cholesterol, often referred to as "bad" cholesterol), and triglycerides. These changes, when combined with the significantly longer lifespan for those on HEMT, can potentially elevate the risk for cardiovascular disease (CVD).

The key to managing this emerging risk is early detection, which allows for timely interventions, such as dietary adjustments or medication, before heart disease develops. In fact, the American Academy of Pediatrics (AAP) strongly recommends universal cholesterol screening for all children between ages 9 and 11 years and again between 17 and 21 years. Based on the emerging evidence in CF, your CF Team has elected to add a blood lipid panel to your annual labs per the AAP guidelines. To help us interpret these results, we have partnered with Dr. Amy Levenson from UNC Pediatric Endocrinology, who is an expert in managing lipid disorders in children and is the Director of the Pediatric Lipid Clinic. She will be working closely with your CF care team to review your lipid panel results, provide expert interpretation of any changes or abnormalities, and recommend appropriate therapeutic interventions, including lifestyle changes and medications when indicated.

As always, your CF team is happy to discuss this new screening process and your overall health at your next visit.

Celebrating Excellence in Care

We are thrilled to share exciting news that following a successful site visit by the Cystic Fibrosis Foundation earlier this spring, the UNC Pediatric CF program was honored with the CF Foundation's Quality Improvement Award: Recognizing Outstanding QI Processes and Accomplishments at the 2025 North American CF Conference.

This recognition reflects our ongoing commitment to continuously working to improve the care for children and families living with cystic fibrosis. We are grateful for your trust and partnership in this journey—your feedback and collaboration make achievements like this possible.

Thank you for being part of our community as we continue working together to improve health and quality of life for every child in our program!

Pictured (left to right): Alannah Mascarella, Kimberly Stephenson, Cameron McKinzie, Whitney Brown (CFF), Charles Esther, Jerry Nick (CFF), Tonya Stafford, Charissa Kam, Paige Noreen, Kaitlin Tillman



Newborn Screening Updates



UNC has partnered with the North Carolina Department of Health and Human Services and Duke University to try to improve newborn screening (NBS) for CF across the state. With the partnership of several parents of children with CF, we learned that some families have received automatic notifications of a newborn screen concerning for CF even before their pediatrician was informed. This is due to the 21st Century Cures Act, a federal law that requires immediate release of test results via the electronic health record. Results are released to the patient portal without any context or explanation. This is particularly concerning in CF, where NBS is a two-stage process.

In the first step of CF NBS, the baby's dried blood spot is tested for a substance called IRT, or immunoreactive trypsinogen. This can be an early marker of CF, though other conditions including premature birth or a stressful delivery can also lead to a high IRT. The State Lab will flag the top 4% of IRT values for the day and send those blood spots on for additional testing. The parents are notified at this point of an "abnormal" NBS for CF.

The second step of CF NBS involves analyzing that same blood spot for genetic markers for CF. This is also done at the State Lab and takes about 10 days to complete. Only a very small number of babies with the high IRT value will show genes that are suggestive of CF. Babies who have a high IRT value with CF-causing genes present are referred to a CF center for sweat testing, while most babies with a high IRT value and no CF genes present do not need to have any further testing done.

Our team has created a flowsheet explaining this process to parents, and we are working to get it uploaded to patient portals when the first step IRT result is uploaded. We hope that this provides more information and context for families regarding CF NBS. We are also working on streamlining the information that is given from the State Lab to the pediatricians when NBS testing is completed. We have worked together to make the referral process to a CF center easier and provided clear "next steps" for pediatricians who may never have handled a positive NBS result for CF. Importantly, we want pediatricians to know that CF can affect babies of all races and ethnicities, and sometimes the newborn screen can give falsely reassuring results to babies who are in a racial or ethnic minority. If there are clinical signs or symptoms of CF, the CF care team at UNC is glad to help these providers decide what is the right way to evaluate these children for CF.



Legal Resources

Given UNC's recent issues with Cigna Insurance coverage, we wanted to remind our CF patients about the COMPASS resource that the Cystic Fibrosis Foundation offers. They are very helpful with any insurance questions you may have. You can reach them at 844-COMPASS or compass@cff.org.

RESEARCH



REACH: An observational study for people with CF who do not take CFTR modulators, ages 12+. Researchers will use the health information collected from this study to improve medical understanding of CF and to advance new therapies for people with CF. There is an accompanying website with more information at reachcfstudy.com.

STOP-PEDS: Study to evaluate two antibiotic treatment approaches for the management of outpatient pulmonary exacerbations in children 3 to 18 years old, Ages 3-18; The STOP Peds study will evaluate the safety and effectiveness of two antibiotic treatment approaches for pulmonary exacerbations in children with CF. Those in the immediate antibiotics treatment arm will be prescribed 14 days of oral antibiotics. Those in the tailored therapy treatment arm will only begin antibiotics if they meet certain criteria, such as their symptoms worsen or do not improve over time.

SUN-CF: Screening for Unmet Needs in Cystic Fibrosis; Reach out to Caroline Flowers for more information.

F19 K23: 19F MRI in Healthy Children and Children With Mild Cystic Fibrosis Lung Disease ([clinicaltrials.gov NCT06066723](http://clinicaltrials.gov/NCT06066723)), Ages 6-17; Children and adolescents (6-17 years old) with cystic fibrosis (CF) who have normal spirometry will undergo 19F MRI with the inhalation of an inert contrast gas to study ventilation. Comparisons will be made to a cohort of healthy children (6-17 years old) who will perform the same measures. The primary outcome measure is the feasibility of conducting these studies in the pediatric population.

Mental Health Risk and Resilience in Caregivers of Children with Cystic Fibrosis, legal guardian and caregiver of a child with CF (between 2-12 years of age), This study involves completing a 30-45 minute questionnaire on a secure website and allowing access to your child's medical record to collect de-identified medical information. Participants will receive a \$40 electronic gift card. If you would like to learn more, please contact Kelly Moormann at kelly.moormann@med.unc.edu.

Sinus Disease in Young Children with CF, "Do you have a child with cystic fibrosis under 11 years old? We invite you and your child to participate in a research study led by Dr. Jane Gross to help understand how CF affects the sinus and sense of smell. Participants will complete a quick sinus MRI scan and some surveys once a year for 5 years. Each visit will be compensated for \$50 and reimbursed for travel costs. Contact Mia Sharrock (mia.sharrock@unc.edu / 919-445-4788) for more details."

TIDES 2.0, a national study aimed at understanding the well-being of children with CF. Children 2-11 years of age will be strategically enrolled so investigators can learn more about depression, anxiety, and behavior problems in children with CF and how these issues change over time. Investigators will also study side effects related to CF medications.

CF Nontuberculous Mycobacteria (NTM) Research

Nontuberculous mycobacteria (NTM) are bacteria that live in soil and water and can cause chronic pulmonary infection in people with cystic fibrosis (CF). These infections may lead to severe lung disease and are often very difficult to treat. The most common infections are caused by *Mycobacterium abscessus* complex and *Mycobacterium avium* complex. People are exposed to NTM infections on a regular basis, but only a small percentage of people will develop NTM lung disease. Dr. Gross's lab is interested in understanding the sources and pathways of how NTM infect people with CF. She is the principal investigator of the Prospective Healthcare-Associated Links in Transmission of NTM (HALT NTM) Study to identify and mitigate potential NTM infection outbreaks at CF Care Centers. Specifically, this study identifies NTM respiratory isolates collected from people with CF and compares the respiratory isolates to each other as well as NTM isolates recovered from dust and water biofilms in the healthcare environment. This study will help us better understand the mechanisms that cause NTM infections in people with CF.

CF *Mycobacterium abscessus* Research

Dr Gross is the site PI for a study called POSTSTAMP, A Prospective Standardized Assessment of People With Cystic Fibrosis and Non-tuberculosis Mycobacteria Pulmonary Disease Undergoing Treatment With Mycobacteriophage. About 10 people with cystic fibrosis (CF) and persistent Nontuberculosis mycobacteria (NTM) infection despite treatment will be screened to find out if their NTM infection has at least one mycobacteriophage that is effective in killing the mycobacteria. Individuals who are found to have at least one phage will be offered assistance in pursuing FDA approval for treatment via expanded-access Individual New Drug (IND) for compassionate-use. They will receive phage treatment for 1 year along with their guideline-based antibiotics for NTM. Individuals who are not identified as having a phage match will be followed as they continue to receive guideline based antibiotic therapy for 1 year. All subjects, including those who do not have a phage match will continue to be observed for the duration of the study, or about 1 year.

If you'd like to learn more about research in general or may be interested in discussing any of these research studies, please reach out to Caroline directly at caroline_flowers@med.unc.edu or 984-974-2962.

The STORM CF Study Results

STORM-CF (Streamlining Treatment Or Reducing Medication) is a study that looked if therapies aimed to reduce the mucus burden (mucolytics) like Pulmozyme (dornase alfa) and hypertonic saline can be safely stopped for people with CF who are on Trikafta.

The study was done in the UK and enrolled people who had been on Trikafta for at least 3 months, were 6 years old and older, and had a lung function above 40%. They randomly assigned them to stop or continue their therapies and followed their lung function at each clinic visit using the UK CF registry data and followed their lung function for one year.

The majority of people (about 70%) were on Pulmozyme alone, around 27% were on both Pulmozyme and hypertonic saline, and 4% were only on hypertonic saline. At the end of the year, the difference in lung function was minimal between those that continued versus stopped these therapies (about 1%). They similarly found no significant change in lung function in those who stopped treatments versus those who continued treatments when they analyzed by age group and severity of lung disease. There are more detailed analyses that will be done in the months to come such as looking at which therapy (Pulmozyme or hypertonic saline) made a bigger difference.

Overall, STORM-CF results are similar to what had been shown in the “SIMPLIFY” trial that was much smaller in scale and only followed participants for 6 weeks. Both studies’ results indicate that it may be safe to stop some or all mucolytics. Yet, it may still be important to do airway clearance by some measure – for example, exercise.

As always, talk to your Care Team if you have any questions about this study or your child’s therapies.

Contact Us

Scheduling: 984-974-7337
(En Español: 919-966-6669)

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

Hospital Operator: 984-974-1000

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



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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.