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CYSTIC FIBROSIS CENTER NEWS & NOTES

SPRING 2021, VOL. XXXV

NEWS FROM THE FRONT

Covid-19 and Cystic Fibrosis

By Samya Nasr, MD, Director, Cystic Fibrosis Center

COVID-19 is caused by SARS-CoV2 (severe acute respiratory syndrome coronavirus2). With the growing COVID-19 pandemic, we need to understand its impact on patients with cystic fibrosis (CF). As we all know, the lockdowns started in March, and virtual clinic visits began. All the steps taken to decrease the spread of COVID-19 have resulted in reductions in face-to-face clinic visits, clinical measurements and pulmonary exacerbations. In May, the International COVID CF Registry published their first report, and it showed that a more severe presentation of the virus may be associated with older patients, CF-related diabetes, lower lung function in the year prior to infection and having received an organ transplant. The outcomes in this large study are better than initially feared overall, possibly due to a protective effect of the relatively younger age of the CF population compared to other chronic conditions. However, COVID-19 is not a benign disease for all people in this patient group; it can be asymptomatic or cause severe disease and can affect virtually any organ.

COVID-19 symptoms include fever, cough, headaches, new shortness of breath and difficulty breathing, muscle pain, chills and new loss of taste or smell. Other symptoms include: sore throat, diarrhea, malaise, fatigue and loss of appetite. Risk factors include hypertension, obesity, diabetes, cardiovascular diseases, chronic pulmonary diseases, chronic kidney diseases, malignancy and chronic liver diseases. Risks are increased for minority racial and ethnic populations in the US, likely due to poor access to health care and inability to stay home because of the need to work. Patients who contract the virus can have pneumonia, acute respiratory distress syndrome, acute liver injury, cardiac damage and dysfunction, thrombotic events, acute kidney injury, neurologic manifestations and shock. Generally, milder symptoms show in the upper respiratory tract only and rarely require hospitalization. Less than 5 percent of laboratory confirmed cases are less than 18 years of age.

Summary of publicized treatment options:

1. **Hydroxychloroquine:** does not help and may cause harm.
2. **Remdesivir (antiviral agent):** originally developed for hepatitis C virus then tested against Ebolavirus. It showed effectiveness against SARS- CoV and MERS- CoV in animal models. It shortens time to recovery in moderately ill patients.
3. **Steroids:** Reduces 28-day mortality in patients who require supplemental oxygen.
4. **Convalescent Plasma from patients who had COVID-19:** Has been tried in hospitalized patients and has been reducing symptoms of the disease.
5. **Two monoclonal antibody therapies (bamlanivimab and casirivimab + imdevimab):** have been given FDA EUA approval and they have been given to some non-hospitalized adults and adolescents (12-17 years old) with mild to moderate symptoms of COVID-19 who have risk factors for progression to severe disease. The supplies are very limited, though, and the focus is on severely immunosuppressed patients.

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NEWS FROM THE FRONT

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Vaccines

A single set of FDA regulatory requirements for licensure applies to all vaccines, regardless of the technology used to produce them. They should be manufactured, processed and packed to meet standards designed to assure that the biological product continues to be safe, pure and potent. Also, accelerated approval is based on adequate and well-controlled clinical trials. Each vaccine sponsored by the US Department of Health and Human Services' Operation Warp Speed uses a slightly different approach with the same goal: to induce an immune response in the body against SARS-CoV2, the virus that causes COVID-19. The vaccines produced by Pfizer and Moderna are both mRNA vaccines.

Only those COVID-19 vaccines that are demonstrated to be safe and effective, and that can be manufactured in a consistent manner, will be licensed by the FDA.

On December 11, 2020, the US Food and Drug Administration (FDA) granted Emergency Use Authorization (EUA) to one COVID-19 vaccine produced by Pfizer Inc./BioNTech. The vaccine produced by Moderna was approved by the FDA shortly thereafter, on December 18, and received the EUA.

The Pfizer and Moderna vaccines both contain:

- mRNA – the main active ingredient that elicits an immune response and the production of antibodies
- Lipids (including ((4-hydroxybutyl) azanediyl)bis(hexane-6,1-diyl)bis(2-hexyldecanoate), 2 [(polyethylene glycol)-2000]-N,N-ditetradecylacetamide, 1,2-Distearoyl-sn-glycero-3- phosphocholine, and cholesterol) — an outside coating or shell of fat that protects the mRNA from destruction as it is being stored, administered and delivered to cells
- Potassium chloride; monobasic potassium phosphate; sodium chloride (salt); dibasic sodium phosphate dehydrate — salts that are used to maintain proper levels of acidity (pH)

This type of vaccine operates somewhat differently than the seasonal flu vaccine and some other types. mRNA vaccines contain a message from the virus that causes COVID-19 that gives our cells instructions for how to make a harmless protein that is unique to the virus.

After our cells make copies of the protein, they destroy the genetic material from the vaccine. Our bodies also recognize that the protein should not be there and build immune cells that will remember how to fight the virus that causes COVID-19 if we are exposed in the future.

Most of the vaccines will require two shots, with the second shot received 21 to 28 days after the first, depending on the particular vaccine. Johnson and Johnson's vaccine received EUA and FDA approval February 27, 2021. It is a single shot vaccine, which transports the message from the virus in an inactivated cold virus. The end product is the same as the mRNA vaccines, with our cells making copies of the protein to build immune cells.

Available vaccines that have completed phase 3 trials and that the FDA is evaluating for approval:

- Protein Based vaccine: Novavax NVX Co-V2373
- Viral vectors: AstraZeneca AZD 1222

As vaccine development progresses, the CF Foundation and our center continue to engage federal and state decision-makers to ensure they understand the needs of the CF community. People with CF (ages 16 and older), may be eligible to receive a vaccine based on their health status in consultation with their doctor. Vaccine distribution is determined by the Centers for Disease Control and Prevention (CDC). Initially, there will be a limited supply of authorized vaccines. When a vaccine becomes available for CF, you will likely have only one option. According to CDC, vaccination should be offered to persons regardless of history of prior symptomatic or asymptomatic SARS-CoV-2 infection. Current evidence suggests that people who have had COVID-19 may be protected for up to 90 days after their initial infection, so they may decide to wait until after this period, if desired.

My Career in Cystic Fibrosis: *Past, Present and Future*

By Richard H. Simon, MD, Adult Program Director

I am retiring in July, and have been reflecting on the amazing progress I have witnessed in the treatment of cystic fibrosis (CF) during my career. My intent in sharing my thoughts is not just to reminisce, but to show how the strategies we have used to get to where we are today will work to bring us to an even better future for those with CF.

My start in CF occurred when I was a medical student at Duke University in the early 1970s. I was assigned to a pediatric ward where there were two young patients with CF. As part of my learning, I tried to read everything I could about CF, but what I found was very disappointing. Even to a green medical student, I could tell that the understanding of CF lung disease was dismal and that the available treatments were marginally effective at best.

Later as an adult pulmonary fellow at the University of Colorado, I was asked to be the "CF Fellow," which meant that I would participate in the care of adults with cystic fibrosis who received their care there. At that time in the US, the small number of adults with CF were usually cared for by pediatricians. But the head of my pulmonary division believed that adults should be cared for by adult trained caregivers. When I moved to the University of Michigan in 1981, I was lucky that my new division chief was also one of the few leaders who advocated for adults with CF to be seen on adult services. Because there were so few other programs that held a similar view, I became one of a small number of adult pulmonologists to learn how to provide CF care.

Fortunately, the 1980s saw important advances that for the first time gave us a deep understanding of CF. Paul Quinton, a scientist who incidentally also has CF, discovered that the fundamental problem in CF is the loss of an anion channel that allows chloride to move into or out of cells. And in 1989, Francis Collins at the University of Michigan with colleagues at the University of Toronto located and characterized the CF gene. I cannot emphasize enough the importance of these two discoveries. They set the foundation for all the great advances we are seeing now and will continue to see into the future.

In the 1990s and 2000s, there was a steady introduction of new pulmonary therapies including dornase alfa (Pulmozyme®), inhaled antibiotics, inhaled hypertonic saline and azithromycin. Each one improved the health of patients by small but significant amounts. A really major advance occurred in 2012 when the CF modulator, ivacaftor, was approved providing the first highly effective therapy for CF. Sadly, it helped only a small number of patients based on their CFTR mutations. But this discovery set the stage for what is clearly the most amazing advancement I have witnessed in my career, namely the introduction in 2019 of the triple combination, elexacaftor/tezacaftor/ivacaftor (Trikafta®). This extraordinary treatment will bring highly effective CFTR modulator therapy to 90 percent of people with CF.

It is important to point out that these treatments weren't discovered by accident. They were developed by innovative scientists applying state-of-the-art techniques with support from the Cystic Fibrosis Foundation, the National Institutes of Health, donors, and patients with CF. And we are not done. The same strategies that have led to our current state will carry us to the next breakthrough and eventually to a cure.

On July 1, I will become an active emeritus professor and curtail my clinical activities and pass my administrative duties to others. I will continue my work with the CF Foundation, including their Data Safety Monitoring Board that oversees clinical trials of CF treatments. I am fortunate that our adult program has an exceptionally strong and experienced group of caregivers who are already doing the bulk of the work. Dr. Shijing Jia and Dr. Tom Sisson will be co-medical directors of the adult program. Both have extensive experience with CF clinical care, and Dr. Jia has been very active in CF clinical research and in quality improvement efforts. I will greatly miss the wonderful relationships I have had with my patients and their families. But I am comforted knowing that our CF team will ensure that the tradition of outstanding patient care will continue unabated at the University of Michigan.

WHAT'S NEWS

Care Beyond the Pharmacy Counter: *The Role of Clinical Pharmacists and Pharmacy Technicians in CF Centers*

By Hanna Phan, PharmD, FCCP, FPPA

The U-M Adult and Pediatric CF Centers have been fortunate to grow with added expertise in medication effectiveness, safety and access. This expansion involves the addition of clinical pharmacists and pharmacy technicians in the adult and pediatric CF centers.

Clinical pharmacists complete a doctor of pharmacy (PharmD) degree as well as specialized postgraduate training like a residency and/or fellowship.

Clinical pharmacists in CF Care:

- assess and manage the medication regimen, including dosing, drug interactions, drug allergies, side effects
- monitor lab tests related to medication effectiveness and safety
- provide advice and information about medications and supplements
- help develop medication plans with patients and families to help sustain daily care
- address patient and family questions and concerns about medications

You'll usually see the clinical pharmacist before as well as between CF clinic visits, either in person or as a virtual/phone visit. Visits with the PharmD can include a preliminary symptom assessment (so they can assess how well medications are working and possible side effects) and comprehensive medication review, including any herbals or supplements (so they can assess for possible drug interactions). They welcome questions and are happy to be your partner in developing individualized medication plans.

Pharmacy technicians complete a certification program and/or associate degree with specific training related to preparation of medications and navigating insurance and patient assistance programs.

Pharmacy technicians in CF care:

- provide support regarding medication patient assistance programs
- initiate, process and complete medication prior authorizations
- verify prescription insurance benefits
- provide support for troubleshooting prescription insurance issues
- provide support for medication access issues

You may encounter the pharmacy technician at various times. They may be calling to obtain an updated medication list in preparation for a future clinic visit, or contacting you about a medication prior authorization or patient assistance program. They are happy to help with your medication access needs and help answer questions you may have regarding prescription insurance issues or patient medication assistance programs.

Our Clinical Pharmacists and Pharmacy Technicians Team

PEDIATRIC CENTER

Clinical Pharmacists
- Hanna Phan, PharmD, FCCP, FPPA
- Ashley Sabourin, PharmD

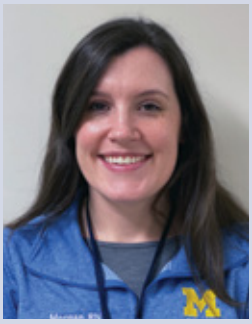
Pharmacy Technicians
- Nichole Culter, CPhT
- Chrita Marshall, CPhT

ADULT CENTER

Clinical Pharmacist
- Linda Fitzgerald, PharmD, BCPS

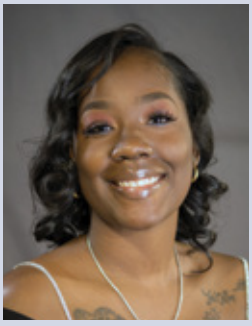
Pharmacy Technician
- Sara Gentner, CPhT

Staff Introductions



Morgan Benson, BSN, RN joined the Pediatric Pulmonary Division in March 2020. She earned her BSN at Eastern Michigan University and has five years of inpatient pediatric nursing experience on units 12 West and 12 East (the pediatric CF home unit). She enjoys continuing to work with the CF population on an outpatient basis and is especially interested in CFRD (cystic fibrosis-related diabetes).

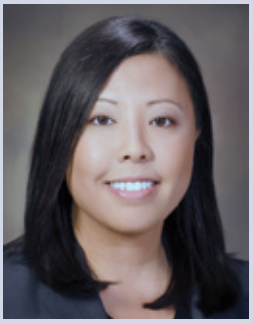
Nichole Culter, CPhT has joined the Pediatric CF clinic to focus on prior authorizations. She spent 12 years with an independent pharmacy and then joined the Transition of Care team here at Michigan Medicine, doing bedside delivery of discharge medications.



Chrita Marshall, CPhT is a pharmacy technician in the Pediatric CF Clinic with 10 years of experience in the field, and joins us part time. Her role includes prior authorizations and assisting the pharmacists with CF-specific assessments and projects. She also works

in the ER at Beaumont handling medication reconciliations/medication histories.

Stacey Fogarty-Brown, RD, CSP is a registered dietitian joining the CF team full-time this year. Stacey has been a registered dietitian for five years, with a wide range of expertise in pediatrics, including caring for patients with CF.



Hanna Phan, PharmD, FCCP, FPPA is a clinical pharmacist specialist for the Pediatric CF Center and a clinical associate professor at the U-M College of Pharmacy. Fifteen of her 17 years of experience were focused on CF and other pulmonary diseases like severe asthma, with 12 years at the University of Arizona CF Center. She received her Doctor of Pharmacy degree from U-M and completed a postdoctoral fellowship in pediatric pharmacotherapy from The Ohio State University and Nationwide Children's Hospital.

Natalie Zedro, BSN, RN is the new Adult CF Program co-Coordinator. She is a U-M School of Nursing graduate and has previously worked inpatient on 8D, which was a Moderate Care floor. Natalie has worked with CF and interstitial lung disease patients in the Pulmonary Clinic at Taubman for four years before transitioning into her new role.

CF Conversations: *Adult Peer Support*

CF Conversations is a peer support group open to all of our adult patients with cystic fibrosis at U-M. The group meets every three weeks via Zoom video conference and is usually about an hour long.

CF Conversations was started to allow people with CF that we follow in our center to have an opportunity to connect with one another during the COVID 19 pandemic. Although the group is facilitated by one of our clinic social workers, there is no specific agenda for the group.

The folks who attend introduce topics and questions that are on their minds, and the conversation flows from there. Recent topics of conversation have included coping during the pandemic, managing anxiety, making good decisions about what is safe to do at this time, and how COVID has impacted CF patients' children. There have also been discussions about Trikafta® and its impact, sharing of experiences living with CF and stories of accomplishment and hope.

Periodically, one of our CF physicians will join the conversation for the first 15 minutes and answer questions from participants.

No signup is required: people join the call as their schedules and interests allow. CF Conversations is a casual, yet effective way for Adult CF Center clients to connect with one another and find affirmation and support. Adult Program patients can find the link in their portal. Pediatric Program adult patients can email Mari Pitcher, LMSW for the link if interested: pitcherm@med.umich.edu

CF Learning Network

The CF Learning Network (CFLN) is a collaborative group of accredited CF centers that use quality improvement methods, timely data capture and reporting to test innovations in CF care. The U-M Pediatric CF Program joined the CFLN in 2020, with our first focus being on telehealth. As a group and as individual CF centers, we worked to improve interdisciplinary care and patient/parent visit agenda settings with fully virtual and hybrid (part virtual, part in-person) clinic visits. Creation of our clinic leadership team included a physician leader, Dr. Nasr; quality improvement leaders, Dr. Filbrun and the program coordinator Cathy Enochs; and a parent/family partner Rebekah Raines. With the help of our interdisciplinary team members, we have reached our goals and have maintained them. We're looking forward to continuing this work and engaging with CFLN and the greater CF community to share tested and proven care models. This work directly affects the clinic interactions and your CF care for the better.

PATIENT SPOTLIGHT

Unconventional Dad

By Matt Ozinga, Adult with Cystic Fibrosis

Raising a family with cystic fibrosis makes me feel "normal" in what is far from a normal life.

I arrived at my family in what could be considered a nontraditional sense. My future wife already had a daughter of her own, which had its own complications and yet awarded me the opportunity to be a father—something I thought was an impossibility after learning of the fertility issues that come with CF. We never wanted it to end there, though. It was always our hope to have a large family. Our family planning was unique. Already aware of the probable infertility issues, we went straight to a specialist when it came time for us to try for more kids. A simple lab test was all that was needed to learn we were destined for in vitro fertilization (IVF). Before we committed to the cause, we wanted to make an informed decision on the risks of our kids inheriting CF. After a simple blood test, it was confirmed that my wife was not a carrier of the most common mutations of the disease, and that the risk was minimal in case she carried a very rare mutation.

After several IVF attempts, we welcomed our first son. I was finally able to stop prioritizing my own care (don't tell my doctors), and was able to obsess over the care of someone else for a change. From diapers, feedings, schoolwork, sports practices, sibling rivalries and boo-boos — it was all a healthy and wanted distraction from the woes of CF. It doesn't mean I quit taking care of myself altogether. My kids became my motivation for staying healthy, but my own health didn't take the top spot anymore. Too often, I find myself reading bedtime stories with my 7-year-old daughter and succumbing to my own sleep deprivation, missing nighttime medication doses. Or too often, I'm asked to play video games with my 9-year-old son during times that I had previously dedicated to runs on the treadmill...or chasing after twin 5-year-old boys gets the better of me and I am too mentally drained to stir up motivation to perform my airway clearance.

Before kids, when I was just a kid myself, I remember nights at home when I would fall asleep on the couch before completing my chest PT. I remember stirring awake to my mom strapping my vest on me and cranking the frequency up at the prescribed intervals, and all the while I just continued humming along, sleeping. She'd never let me miss a treatment. Here I am now, an adult with my own kids, feeling guilty of wasting the effort my mom put in all those years keeping me healthy. It's a lot harder to take care of myself these days. But raising a family with CF reminds me a bit of the in-flight safety procedures you hear on an airplane: "Should the cabin lose pressure, please place the oxygen mask over your own mouth and nose before assisting others." If I'm unable to breathe, how on earth am I going to be able to assist with my family's needs? I've learned to be the unconventional dad, making funny noises while wearing my therapeutic vest, or using my IV pole as a limbo bar.

Life these days revolves around what's best for my family. Often, that includes medications, physical therapy, doctors' offices and occasional hospital visits, atypical for most families. But it also includes happiness, unconditional love, real struggles and a lasting commitment to the family unit. These are experiences that resonate with everyone raising a family, and it makes me feel normal.

I Willingly Accept the Challenge

By Betsy Ventura, Adult with Cystic Fibrosis

"What do you want to be when you grow up?" an adult with a friendly smile would lean in and ask. As a little girl, I had but one dream in my heart. "I want to be a mother!"

That dream never faded, and at age 33, after a smooth pregnancy, my dream came true when Lucas arrived. Just following his birth, the doctor placed him on my chest. He held my gaze for several moments; moments full of wonder and joy for me. I was a mother! I became pregnant with Alena seven years later. My disease had progressed and sustaining the pregnancy pushed my body to its furthest limits. It was a marathon, and I am forever grateful to the team of health professionals who helped me through. Alena was born three months shy of my 41st birthday. Although she greeted me with complaints rather than wide eyes, there was no less joy or wonder at the miracle of her arrival.

Cystic fibrosis complicates everything, and motherhood is no exception. Caring for young children soaks up a great deal of time and energy. Treatments, respiratory therapy, exercise and other aspects of managing the disease also demand much. Inevitably, caring for oneself gets crowded out when there isn't space for both.

CF brings unique emotional challenges to motherhood. After my son was born, I began to think about dying. Although I wasn't at a critical stage in my disease, I feared leaving him without a mother. I felt guilt at how my disease was affecting him. It was hard to see the disappointment in his eyes if I was too sick to play. It hurt to miss out on adventures he had with others when I needed to rest. I could hear the worry in his voice when he asked how long it would be until I was better.

Along with these challenges there have been many blessings. I learned early on that having CF meant I needed support to raise my kids. We have been on the receiving end of much love and generosity as friends and family have helped us, and accepting this help has created deep and satisfying relationships that have benefited us all. The bonds of marriage, family and friendship are strengthened when we open up our lives to each other.

One morning, years ago, I was upstairs when a coughing fit overtook me. Lucas, only two years old, left his toys downstairs, and within moments was standing before me holding out one of my inhalers. He watched me use it, then satisfied, went back to playing. In the years following, Lucas often came to my appointments, whispering to me that he would hold my hand if I got scared. If I missed an outing due to illness, he would bring back a little token so I wouldn't feel left out: a muddy stick, a colorful leaf, a dandelion wilting in his purposeful grip. I am sorry that he has seen me suffer and that he has been anxious and worried, yet I am grateful that through those struggles, he has developed a kind and compassionate heart. Alena is not yet two years old, but I hope that she, too, will learn that compassion.

Being a mother has not made my life easier. Yet I willingly accept the challenges: the sleepless nights, the exhausting days of parenting through illness, the worries and frustrations and all the "what ifs." It is well worth it for the joy, the laughter, the love and the blessing of a full and happy life with these two small people who call me Mom.

PARENT TO PARENT

Adapt and Overcome

By Heather Trammell, Parent of a Child with Cystic Fibrosis

As a mother of a little boy with CF, navigating everyday life can be tricky enough. Am I making the right decisions, or is there something more I could be doing? Then comes 2020 bringing a new challenge—a pandemic, adding even more concern, doubt and fear to my already full plate. My approach turned out to be much different than my husband's, exposing new challenges in how we make decisions as a unit.

My husband and I will be married for 10 years this June, and prior to COVID had found our way to communicate and navigate tough waters relatively easily. In full transparency, cystic fibrosis and then COVID have shown us areas we needed to work on, but they have bettered us for the long haul.

My approach to COVID was lock the door, throw away the key, we will see you all on the other side. I was not going in public at all. My thoughts were if we could shield him from it all, then he is most protected. Doing that became isolating, as I am sure others have felt. I could not see through my fear to find balance. Balance that my son, my husband and I myself needed. My husband's approach was different. He felt we should still try and maintain some sort of normalcy and still live our lives but protect our son. We found ourselves butting heads because we both strongly felt our way was best.

This summer we both decided we needed to approach our decisions in a different way. We sat down, as many have, and laid our thoughts, fears and opinions on the table. Neither one of us was wrong and both of us had one common goal: our son's health, safety and well-being. What we have found to work are three things: We hear each other out—fully, really listen; we weigh risk versus reward; and we compromise.

For instance, this past June our best friends were camping and invited us out for the day. My first instinct was NO, too many people, too much possible exposure. My husband felt that it was not too risky, we were with people we trusted, we were going to be outside and could socially distance to the extreme. We had been cooped up all spring and needed a break. My husband pointed out to me all the ways we could protect our son, but still go. We then weighed out the risk versus reward. An approach we have taken to decision-making with our son since the day we brought him home. After deciding that the rewards far surpassed the risk, we went. There were compromises though: I wanted us clear of any crowds and really to hang at the site. My husband understood and backed me 100 percent. We went and our boy had the best day! Sandcastles, hot dogs, digging in the dirt and living his absolute best two-and-a-half-year-old life.

After that first outing, we were able to adopt this approach in all decisions we made concerning our son. We were able to confidently make the best decision for him and us the best way we know how. These were new tools for our communication tool belt. As a result, we confidently and safely went to a petting zoo, fireworks and most recently saw Santa. We both felt good about our decisions and how we got there.

No relationship is perfect, and we all have our challenges. I wanted to share what I have learned during the pandemic to help others, possibly share new tools with you and your spouse, partner or loved one. We are all doing our best to navigate an unprecedented situation to the best of our abilities. Just like our incredible children, we too are strong and can adapt and overcome!

RESEARCH UPDATES

The CF Foundation's Therapeutics Development Network (TDN) is a driving force in CF research. Michigan Medicine is a CF TDN Center, which helps us get involved in clinical research so we can contribute to making improvements in CF treatments and therapies. However, we can only accomplish that with the participation of our patients.

If you have questions about our research program, you may contact Marisa Linn at mlinn@med.umich.edu and Dawn Kruse at dmkruse@med.umich.edu.

In order to help you better understand some of the studies open to enrollment, below are brief summaries of research we are conducting at Michigan Medicine.

Antibiotic Studies:

1. STAR-ter: cycled antibiotics for eradication of new cases of MRSA (recruiting ages 2-45)

Anti-inflammatory Studies:

1. APPLAUD: Use of LAU-7B to reduce inflammation in adults (recruiting ages 18 years+)

Modulator Studies:

1. VX18-445-110: open-label extension for participants in the VX18-445-104 study (Enrollment closed)
2. VX18-445-113: open-label extension for participants in the VX17-659-105 study (Enrollment closed)
3. RESTORE-CF: A Phase 1/2, Randomized, Double-Blind, Placebo-Controlled, Combined Single and Multiple Ascending Dose Study Evaluating the Safety, Tolerability, and Biological Activity of MRT5005 (CO-hCFTR mRNA/ICE LNP) Administered by Nebulization to Adult Subjects with Cystic Fibrosis (not yet recruiting)

Observational Studies:

1. SIMPLIFY: removing hypertonic saline and/or Pulmozyme therapies on patients taking Trikafta (recruiting ages 12+)
2. Prospective Study of Cystic Fibrosis (CF) Patients by Lung Magnetic Resonance (MRI) Technology, CT Scan of the Chest and Clinical Measures of Pulmonary Function (Starting to recruit age 6-11 years old))
3. CHEC-OB-17: CFTR Modulated Changes in Sweat Chloride and Outcomes for patients currently taking an FDA-approved CFTR modulator (recruiting all ages)
4. PICC: evaluating the factors influencing PICC line problems during treatment of CF exacerbations with IV antibiotics (recruiting ages 6+)
5. NTM-OB-17: Evaluation of a standardized approach to diagnosis (PREDICT) and treatment (PATIENCE) of nontuberculous mycobacteria (NTM) (recruiting ages 6+)
6. NOPRODCYS0001: Prospective Phase 0 Study to Investigate Pseudomonas aeruginosa and Staphylococcus aureus Bacterial Load, Patient Characteristics and Exploratory Biomarkers in Adult Patients with Cystic Fibrosis or Non-Cystic Fibrosis Bronchiectasis (not yet recruiting)
7. BEGIN: A Prospective Study to Evaluate Biological and Clinical Effects of Significantly Corrected CFTR Function in Infants and Young Children (not yet recruiting)
8. Readiness to Transition to Adult Healthcare and Pre-Transition Experiences Among Parents/Caregivers and Adolescents and Young Adults with Cystic Fibrosis (not yet recruiting)
9. PROMISE: evaluating the effects of CFTR modulators on airway inflammation and microbiology (enrollment closed)
10. GOAL-e²: G551D Observational Study – Expanded to Additional Genotypes and Extended for Long Term Follow-Up (enrollment closed)

Infection Control Studies:

1. Assessing effectiveness of Infection Prevention and Control in Cystic Fibrosis (not yet recruiting)

TEAM UPDATES

The Cystic Fibrosis Foundation – Michigan-Northwest Ohio Chapter holds several fundraising events including Great Strides Walks, Cycle for Life, Metro Detroit's Finest and Evening with the Stars. For more information on how you can get involved in the fight against cystic fibrosis, please call the chapter office at 248-269-8759 or email us at Michigan@cff.org.

Are you interested in opportunities to advocate to state and federal lawmakers in Michigan? Contact Shelly Francis at the CF Foundation Michigan chapter at sfrancis@cff.org for more information.

GREAT STRIDES®
CYSTIC FIBROSIS FOUNDATION

New Virtual Group Nutrition Classes

Courtney Iwanicki, Pediatric Program dietitian, will be leading a group nutrition class quarterly starting in March 2021. The first class, scheduled for March 9, is "Nutrition in Cystic Fibrosis: Promoting Vitamins to VIP Status." Designed for all ages, the class will help to further expand your knowledge about why specific vitamins and minerals are important in CF and where we can find these in our diet.

Current Class Schedule:

- Tuesday, March 9, 2021, 3-4:00 pm
- Tuesday, June 8, 2021, 3-4:00 pm
- Tuesday, September 14, 2021, 3-4:00 pm

Possible topics for future classes include GI Issues in CF and Impaired Glucose Tolerance and Diabetes in CF, but we'd like to hear your suggestions for topics, too. Please call Courtney Iwanicki (phone 734-936-3340) or send her a portal message if you're interested in attending these group classes or have topic ideas.

CFPEERCONNECT

CF Peer Connect is a peer mentoring program for people with cystic fibrosis and CF family members age 16 and older. Through this program, you'll be matched with a peer mentor who has experience with topics that are important to you. Together, you can connect over video, phone, or email.

Visit: cfpeerconnect.com/about

Advisory Board Updates

Both the pediatric and adult program parent and patient advisory boards have been actively involved during the pandemic. With the transition from in-person visits to fully virtual, and back to more in-person visits, their input, ideas and support has been critical to our center changes and we greatly appreciate all their time and effort.

Home Spirometry for the Pandemic and Beyond

By Amy Filbrun, MD, Pediatric CF Program Associate Director

Spirometry is the main tool we use to measure and assess lung function. It measures some basic numbers, including forced vital capacity (FVC—how much air you can forcefully blow out from a full inspiration), forced expiratory volume in 1 second (FEV1—how much air you can blow out forcefully in the first second of exhaling from a full breath), the relationship or ratio of FEV1 to FVC (FEV1/FVC), and on some spirometers, forced expiratory flow between 25 and 75% of expiration (FEF25-75—a measure of mid-expiratory airflow). Children with CF usually start trying spirometry in clinic around 5 years of age, and most children are becoming able to have fairly reliable technique when they are around 6 years old. From then on, spirometry is a routine part of every clinic visit, and is also used during times of illness, both to diagnose exacerbations and to track improvement with treatment, hopefully with resolution and return to baseline.

Over the last several years, devices for measuring lung function at home have been developed. Handheld devices can gather many of the measures we obtain and monitor in clinic from the comfort of your own home. With the onset of the coronavirus pandemic, many clinics needed to quickly pivot and move to virtual visits in order to continue to care for patients on a regular basis while maintaining social distancing and new routines for the use of personal protective equipment (PPE) and cleaning procedures.

Virtual care has been a vital tool during the pandemic, and while it cannot fully replace a face-to-face clinic visit, with the addition of home spirometry we are able to continue to perform essential testing virtually until we are able to do face-to-face clinic visits quarterly again. With the pandemic, the Cystic Fibrosis Foundation (CFF) recognized the needs, and set out to provide home spirometer devices to patients followed in CF care centers across the country. This has allowed CF centers to slowly distribute home equipment to patients able to perform spirometry.

Our center has worked with the CFF to make spirometers available to our patients, and in the Pediatric Center, our Pediatric Pulmonary Lab has taken the lead in setting up virtual training sessions to teach patients and families how to use the spirometers to make reliable measures, and how to upload data via the patient portal in order for your physician to review the data. It is important to learn proper technique for use of the equipment in order to have usable data, and it is necessary to use the equipment routinely when you first receive it to know your baseline.

Weekly testing can help work it into your routine and find your baseline. We ask that patients and families upload the data into the portal once a month and before a clinic visit. This is particularly important so that when you start having symptoms, we can track your lung function and know if you are seeing a decline that would require a change in your treatment regimen, antibiotics or even admission. Similarly, you will want to be able to assess how well you are responding to treatment. While the pandemic has spurred the distribution of equipment, we think you'll see that there are many uses beyond the pandemic for a home spirometer. Long after we have survived the pandemic (and hopefully we are starting to see the light at the end of the madness), we will be able to use these home spirometers for ongoing monitoring. You will be able to send us reports when you are calling to report a change in symptoms, and we will be able to monitor response to treatment from home, saving you traveling to the hospital for repeat testing.

> "We are able to continue to perform essential testing virtually until we are able to do face-to-face clinic visits quarterly again."

If you have received a home spirometer, but haven't yet received training from the Pulmonary Lab, please contact them to do so at your earliest convenience. Please be sure to upload results through the patient portal. If the family member is over 6 years old and has been performing spirometry routinely in clinic and you haven't heard about receiving a device, please feel free to reach out and see if you are on the list or to request one. Happy blowing!

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