MICHIGAN MEDICINE
UNIVERSITY OF MICHIGAN

Cystic Fibrosis Center
NEWS&NOTES

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NEWS FROM THE FRONT

Cystic Fibrosis in Egypt: *Advancing Care*

By Samya Nasr, MD, Director, Cystic Fibrosis Center

It was always thought by the Egyptian Medical Society that CF does not exist in Egypt. One major problem has been the lack of the physicians' awareness of the disease. Addressing that issue began in 1997, first through yearly lectures and workshops at different national and international conferences in Egypt and universities. I also began visiting children's hospitals in Cairo and following CF patients there. In addition, I led a study on a high-risk patient at Cairo University, Children's Hospital in collaboration with a team of Cairo University physicians and Stanford University geneticists. We were able to diagnose 12 out of 60 patients tested. The study was published in 2007. Since then, CF diagnosis has started slowly after Cairo University bought the sweat testing equipment through donations. Once equipment was secured, a small number of patients were diagnosed, but little resources have been available for patient care. A major problem has been the lack of accurate and affordable ways to diagnose the disease across Egypt. Recently, after years of lobbying, the Middle East CF Association (MECFA), through the support from the Cystic Fibrosis Foundation (CFF), placed diagnostic equipment in four university hospitals across Egypt and provided CF diagnostic education and technical support. Another university was granted access to the sweat testing equipment, furthering the reach. Now there are a total of six universities distributed across the country that have accredited sweat testing equipment.

However, CF care in Egypt is not organized. There are no CF care teams. In addition, CF knowledge around the country is still not adequate. There is no patient registry for people with CF in Egypt. A CF registry is required to document the patients' symptoms across the country, in addition to tracking complications and treatment. It is important in helping to improve care, outcomes, and median survival (which is less than 8 years of age).

In 2021, my team and I started to focus on addressing the barriers to providing CF care, with the support of CFF and MECFA, which was made easier by the fact that sweat testing equipment is now more widely available across the country. We started by training the healthcare teams in the universities that received the equipment, not only in CF general knowledge but also in airway clearance techniques and dietary support. Through donations, we were able to bring four vests to the country to be used by the universities (thanks to the Bonnell Foundation). I discussed the limited funding for staff support with each university administration and was promised to have adequate staff support for the CF patients. Providers across the country still would not consider CF when evaluating a patient, since

(*continued on page 2)*

it isn't yet widely known as being present in Egypt, and so would not send a referral for sweat testing on people who may need it. To address this, we empowered the trained physicians by our team to work with different physicians around the country to improve their knowledge and encourage referral for diagnosis and management. Guidelines and protocols for treating people with CF have now been established in collaboration with the Egyptian physicians and accounting for the available resources there. An Egyptian CF registry was created and is now being used to capture data on Egyptians with CF. In addition, their CF Family Advisory Board (FAB) was formed to help with lobbying for resources to battle the disease.

For the next two to three years, the plans are to continue to work on creating CF centers in the four universities we are working with now. Impressively, one of the universities has already started the multidisciplinary approach to treating CF. Another area of focus will be to work on lobbying, with the Egyptian care teams and FAB, the Ministry of Health (MoH) which is responsible for providing and approving of medications in Egypt. Currently, the MoH does not support patient care beyond treatment for acute infections and limited supplies of needed medications. Providing the MoH with registry data would be very important to give adequate and accurate information about cystic fibrosis in Egypt. The goal of lobbying is to provide adequate medications to the people with CF and allowing medications that are not available in Egypt to be approved like CF vitamins, Pulmozyme®, tobramycin solution for inhalation, and most importantly, the modulators especially Trikafta®. The last focus of the collaboration for the next two to three years is to coach the teams on how to do quality improvement (QI) work to follow and assess their progress.

In summary, while CF care is much improved in the U.S. and other developed countries, it is important to focus on CF care in the developing world. By building CF care teams in these countries and empowering them to advocate for their patients, we will change the nature of the disease there. By lobbying for standard medications, including ones like Trikafta, there will be a dramatic difference in the care and lives of Egyptians with CF.

The Bonnell Foundation: *Living with Cystic Fibrosis Is Awarding a $10,000 Fellowship Grant, in Honor of Dr. Samya Nasr.*

The Bonnell Foundation has awarded a $10,000 fellowship grant to Michigan Medicine in honor of Dr. Samya Nasr, who has changed what cystic fibrosis (CF) looks like for patients and families, says foundation founder Laura Bonnell.

Bonnell decided a fellowship was a good way of showing support for Dr. Nasr's work because the demand for pediatric pulmonology care exceeds the supply. Nationally, there is a concern that the pediatric pulmonology workforce will not be sufficient to meet the needs of children with respiratory diseases. This concern is particularly acute in Michigan, which has only 1.26 board-certified pediatric pulmonologists per 100,000 children, below the national average of 1.5. This ranks Michigan 26th nationally in the number of pediatric pulmonologists per capita, despite being 10th in the U.S. in total population and in the number of children. The limited supply of pediatric pulmonologists represents a serious threat to the quality and availability of respiratory healthcare for children with chronic diseases such as cystic fibrosis and asthma. According to a 2017 Children's Hospital Association survey, 48 percent of patients had to wait longer than two weeks for appointments.

For an interview with either Dr. Nasr or Laura Bonnell, please call or email: 248.860.3899 thebonnellfoundation@gmail.com

From left: Nada Youssef, M.D. Pediatric Pulmonary Fellow; Dr. Samya Nasr, M.D. Director, Professor of Pediatrics, Dir. Cystic Fibrosis Center, Laura Bonnell, Founder/CEO of The Bonnell Foundation: Living with cystic fibrosis; Thomas Saba, M.D. Director, Fellowship Program in Pediatric Pulmonology; Carey Lumeng, M.D. Interim Director, Division of Pediatric Pulmonology

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NEWS FROM THE FRONT

New Medications for People with CF: *Beyond Modulators*

By Tom Sisson, MD, Adult CF Program Director

It has been another exciting year in the adult program. We continue to care for an increasing number of patients (now around 350 adults), and our growth can be attributed, in part, to individuals living healthier and longer lives as a byproduct of more effective treatments. Data from the Cystic Fibrosis Foundation reveal improvements in lung function and survival over the past five years, and the availability of highly effective CFTR modulators has had the largest impact on these outcomes. Unfortunately, not everyone is eligible for a modulator, and there are a small number of patients who are eligible but cannot tolerate these medications. For both groups, it is essential that we continue to identify new treatments that reduce symptoms and prolong life. For those individuals who are benefiting from a modulator, it is important that we look for ways to reduce the burden of daily treatment routines. Here, we will review the results of several important clinical trials that inform us about how we can safely limit our patients' exposure to antibiotics and decrease daily medications. We also will provide a brief overview of novel non-modulator treatments currently in development.

The frequency of a course of antibiotic treatment for an exacerbation of lung disease in people with CF has decreased significantly with the approval of Trikafta®. Nonetheless, both oral and IV antibiotics are still commonly prescribed by our clinic, and even though antibiotics are generally well tolerated and have few side effects, we do care for people with CF (pwCF) who have hearing loss and/or kidney insufficiency from antibiotic treatments received over many years. Furthermore, frequent exposure to antibiotics causes bacterial resistance, increasing the difficulty of treating future exacerbations. With the goal of reducing antibiotic exposure, the STOP2 Trial compared short versus long antibiotic treatment courses. In people with CF who improved significantly (as assessed by lung function and a symptom score) by day 7 of therapy, the study found no difference in outcomes (symptoms or lung function) if antibiotics were stopped at day 10 versus day 14. In patients who had not improved by day 7, there was no advantage to extending antibiotic treatment for 21 days versus stopping after 14 days. Based on these results, our adult program Quality Improvement Committee is working to implement a protocol that will decrease the length of antibiotic exposure for our patients. The pediatric program has already implemented this protocol.

In addition to reducing exacerbation frequency, Trikafta also significantly decreases respiratory symptoms including cough and sputum production. This benefit has led many adults with CF to discontinue their inhaled maintenance treatments including Pulmozyme® and hypertonic saline. However, as providers, we have not been certain if stopping these treatments was safe. The SIMPLIFY study was designed to compare the outcomes in persons with CF taking Trikafta who continued versus stopped Pulmozyme or who continued versus stopped hypertonic saline. Participants enrolled in the trial were 12 years and older, with FEV1 of 70 percent or higher, were followed for 6 weeks and assessed for a worsening lung function. The study found that, especially in patients with FEV1 90 percent or higher, there was no difference in lung function (FEV1) with continuing or stopping either of the two inhaled treatments. These results have given the adult physicians in our clinic the confidence to have conversations with our patients about a trial off Pulmozyme or hypertonic saline (or to support our patients who have already discontinued these medications). However, because the trial was short and took place during the pandemic, we are individualizing our recommendations to each patient and fully support those individuals who want to continue these treatments.

The CFF is also committed to developing new treatments that reduce symptoms and lengthen lifespan. These new therapies will be most beneficial for individuals who are not already taking a highly effective modulator. Although considerable effort is being made to 'fix' CFTR through gene therapy and gene editing, there are also ongoing studies to assess new antibacterial medications, drugs that thin mucus, and antiinflammatory medications. One example of a new treatment targeting chronic Pseudomonas infection is AP-PA02, a cocktail of multiple different types of bacteriophages that is administered by inhalation. Bacteriophages are viruses that attack and kill bacteria, and in lab tests, AP-PA02 was able to destroy more than 80 percent of Pseudomonas strains from pwCF. This therapy is now being tested for safety in clinical trials. In the category of drugs that thin mucus, OligoG is a dry powder oligosaccharide (carbohydrate or sugar molecule) extracted from brown seaweed. It has been shown to decrease the thickness of mucus in the lungs and may help pwCF clear mucus easier. Early clinical trials show that OligoG is safe, and we are awaiting studies to prove its benefit.

The care providers in the adult CF clinic have never been more optimistic for the future of our patients. Much of this optimism arises from the availability of highly effective modulator medications for most persons with CF. However, we also remain committed to improving the outcomes of individuals who are not eligible for or cannot tolerate these treatments. Hopefully, in next year's newsletter, I will be able to describe new therapies for these people. Until then, we wish everyone a happy and healthy 2023.

WHAT'S NEWS

Mott 12 East Team Receives Prestigious Beacon Gold Award

C.S. Mott Children's Hospital's 12 East team has a reason to celebrate after receiving the prestigious Beacon Gold Award for Nursing Excellence!

The award is given out by the American Association of Critical-Care Nurses to individual hospital units across the country.

The 12 East team joins only around 150 hospital units nationwide to receive gold designation status — and only three in the state of Michigan.

According to the AACN, "Beacon awardees set the standard for excellence in patient care environments by collecting and using evidence-based information to improve patient outcomes, patient and staff satisfaction, and credibility with consumers. A Beacon Award signifies a positive and supportive work environment with greater collaboration between colleagues and leaders, higher morale, and lower turnover. The process can be driven by the nursing staff, nurturing empowerment and leadership."

Pediatric CF Team Wins Evan Newport HOPE Award in 2022

On December 18, 2022, the Pediatric CF Program at C.S. Mott Children's Hospital was awarded the Evan Newport HOPE Award. The HOPE Award honors staff, faculty, and students who are making a difference in the lives of patients and families by demonstrating a commitment to patient- and family-centered care, and the ideal patient and family experience. The pediatric CF clinic team includes over 50 people dedicated to communicating with and engaging people with CF and their families while providing safe and effective care.

About the Evan Newport HOPE Award

The HOPE Award was developed and designed by Scott Newport and his son Noah — inspired by those who allowed Evan, Scott's son, to have a happy childhood while he was a patient at C.S. Mott Children's Hospital. Not only does Evan's legacy live on at Mott, but his award has given him an opportunity to acknowledge those who work hand in hand with patients and families across the entire health system.

Home Spirometry: *A Research Opportunity*

Our pediatric CF team is investigating whether the frequent use of home spirometry by our children and adolescents with CF can lead to detecting and treating early pulmonary exacerbations. We will also evaluate whether frequent home monitoring and follow-up with people with CF would lead to improved medication adherence. Most of our patient population received home spirometry equipment through the Cystic Fibrosis Foundation to help with their care during the COVID-19 pandemic. This study will look at the impact of frequent use and monitoring of home spirometry on individual overall lung function (FEV1), number of pulmonary exacerbations, and adherence to medication.

This will be a 6-month study that will recruit patients between 6-21 years old (30 patients in the 6-12 year-old group and 30 patients in the 13-21 year-old group). People eligible for the study will need a Forced Expiratory Volume percent predicted (FEV1) of 40 percent or higher. They will be asked to perform home spirometry once a week and upload results into the EPIC portal to keep confidentiality. To assess for adherence, pharmacy refill data for highly effective modulator and dornase alfa (Pulmozyme→) will be obtained from all participants, looking at the six months before the study and at the end of the study. All patients will be compensated $100 at the end of the study for their participation. Patients can opt out at any time.

We predict that the frequent use of home spirometry can lead to improved adherence to medications and help identify early signs of pulmonary exacerbations. This study will begin in early 2023. Our team will connect with you to discuss the study and inquire about interest if you meet eligibility requirements

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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 multicenter clinical research so we can contribute to making improvements in CF treatments and therapies. In addition, our center has been doing our own research as well. However, we can only accomplish that with the participation of our patients! If you have questions about our research program, please contact Marisa Linn at mlinn@med.umich.edu or Dawn Kruse at dmkruse@med.umich.edu.

If you're interested in participating in research, but don't see anything for you here, feel free to browse the CF Foundation's Clinical Trial Finder (https://apps.cff.org/trials/finder). Contact Dawn or Marisa if you see something you are interested in so we can help you navigate the referral to another CF Center to participate in the study (your general CF care will continue to stay with us!).

In order to help you better understand some of the studies open to enrollment, below are brief summaries of research we are currently 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. INS1007-211: A Phase 2a, Single-Blind, Placebo-Controlled, Parallel-Group Study to Assess Safety, Tolerability, and Pharmacokinetics of Brensocatib Tablets in Adults with Cystic Fibrosis **(Enrollment Closed)**

Modulator Studies:

1. VX20-121-102: A Phase3, Randomized, Double-blind, controlled Study Evaluating the Efficacy and Safety of VX-121 Combination Therapy in Subjects With Cystic Fibrosis Who Are Heterozygous for f508del and a Minimal Function Mutation (F/MF) **(Enrollment Closed)**

2. VX20-121-103: A Phase 3, randomized, Double-blind, Controlled Study Evaluating the Efficacy and Safety of VX-121 Combination Therapy in Subjects with Cystic Fibrosis Who Are Homozygous for f508del or Heterozygous for f508del and a Gating (F/G) or Residual Function (F/RF) Mutation **(Enrollment Closed)**

3. VX20-121-104: A Phase 3, Open-label Study Evaluating the Long-term Safety and Efficacy of VX-121 Combination Therapy in Subjects with Cystic Fibrosis **(Enrollment Open)**

Observational Studies:

1. Prospective Study of Cystic Fibrosis (CF) Patients by Lung Magnetic Resonance (MRI) Technology, CT scan of the Chest and Clinical Measures of Pulmonary Function **(Age 6-11 Years Old- Enrollment Closed)**

2. CHEC-OB-17: CFTR Modulated Changes in Sweat Chloride and Outcomes- for patients currently taking an FDA-approved CFTR modulator **(Recruiting All Ages)**

3. NTM-OB-17: Evaluation of a standardized approach to diagnosis (PREDICT) and treatment (PATIENCE) of nontuberculous mycobacteria (NTM) **(Recruiting Ages 6+)**

4. BEGIN: A Prospective Study to Evaluate Biological and Clinical Effects of Significantly Corrected CFTR Function in Infants and Young Children **(Enrollment Paused)**

5. PROMISE: Evaluating the effects of CFTR modulators on airway inflammation and microbiology **(Enrollment Closed)**

6. HERO-2: Home Reported Outcomes in People with Cystic Fibrosis Taking Trikafta **(Enrollment Closed)**

7. Mayflowers: To characterize changes in FEV1 over the course of pregnancy based on cumulative CFTR modulator use while pregnant along with defining other factors that may influence changes in pulmonary function including duration of pre-pregnancy modulator use, baseline FEV1, genotype, history of exacerbations, and pre-existing co-morbid conditions. **(Recruiting)**

8. NICE-CF: A Multi-Center Study of Non-Invasive Colorectal Cancer Evaluation In Cystic Fibrosis **(Recruiting)**

9. NEMO: A Non-Interventional Study Evaluating the Impact of Trifakta Initiation on Children and Caregivers of Children with CF Aged 2-5 **(Not Yet Recruiting)**

Infection Control Studies:

1. Assessing effectiveness of Infection Prevention and Control in Cystic Fibrosis **(Recruiting)**

Phage Studies:

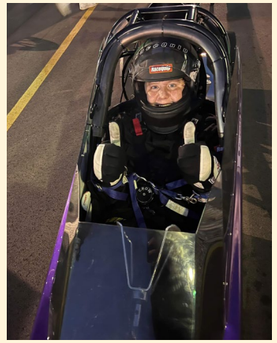
1. 20-0001: A Phase 1b/2, Multi-Centered, Randomized, Double-Blind, Placebo-Controlled Trial of the Safety and Microbiological Activity of a Single Dose of Bacteriophage Therapy in Cystic Fibrosis Subjects Colonized with Pseudomonas aeruginosa **(Recruiting)**

2. Swarm-PA: A Phase 1b/2a, Multi-Center, Double-Blind, Randomized, Placebo-Controlled, Single and Multiple Ascending Dose Study to Evaluate the Safety and Tolerability of AP-PA02 Multi-Phage Therapeutic Candidate for Inhalation in Subjects with CF and Chronic Pulmonary Pseudomonas aeruginosa (Pa) Infection **(Enrollment Closed)**

PATIENT SPOTLIGHT

The "Joys" of High School

By Chase Kulik, a person with CF



Chase, tell me about yourself.

My name is Chase Kulik, and I am a 16-year-old with cystic fibrosis. I am currently a sophomore at Saline High School. My goals are to be a famous chef but, in the meantime, I love cooking, drag racing, and working on cars.

I heard you have some insight on how to deal with high school while also having CF.

Yes, I do. Any teenager will tell you high school is not easy but fighting CF puts it on a whole different level. I have a lot more responsibilities than a normal high school student and I have a lot more challenges. CF has a lot of upkeep required when you are feeling good and a lot more when you are sick. I can't just roll out of bed, throw on some clothes, and head out the door. I need to make sure to do my breathing treatments, arrange my meds in my bag, make sure I have my hearing aids and testing supplies, and plan for any variations in my day. I have spent a lot of time the last couple of years in the hospital and I have learned for me what works and what doesn't so that I do not fall too far behind in school.

With the knowledge that you have gained, what is something you would recommend to other people with CF going to high school?

I think the most important things for me are taking care of yourself, knowing your 504/IEP, self-advocating, and communication.

Taking care of yourself is the most important thing and it means not skipping out on treatments or medications. Being sick and missing a lot of school sucks. If you are anything like me and hate doing homework, this just ensures that you will have a lot to do once you are healthy.

The second important thing to know is your 504 or IEP (plans for the school to support your needs). I look at my 504 as a tool bag that makes sure that I can get through my day with as few issues as possible. For me this covers my digestive issues, breathing treatments, medications, absences, hearing issues, and missing assignments. Like most people with CF, when I am sick or in the hospital the last thing on my mind is school. The 504 makes sure that although I am missing classes, the school will work with me within the guidelines of my 504 to help me catch up. The other key point is that this is an evolving document. Never be afraid or hesitant to ask for something to be included. This tool is made so that you can succeed. This leads me into self-advocating. By knowing your 504/IEP you have the knowledge to ensure that you get the help you need. Not all teachers, counselors, or staff will know these or follow them. It is up to you to make sure that you stand up for yourself. Learn to be comfortable with who you are and what you need to succeed. Until high school I relied on my parents to talk to my teachers and school staff, but I learned that at school I need to speak up. It took me a few tries and a few mistakes but self-advocating and sharing my experiences not only helped myself but also my teachers.

Finally, all of these lead to communication. If you don't keep an open line of communication with your teachers, doctors, principal, and counselor you will struggle to succeed. It is hard for them to understand what it means to have CF and how being sick takes a huge toll on you. When you are sick, you are not missing classes and assignments for one class but for multiples. This means that the workload piles up along with the confusion. This is where communication is important. I learned the hard way as I struggled to catch up after a hospital stay and I relied on my teacher to let me know what I was missing. Unfortunately, by not communicating and reminding my teachers that I was working to catch up, I did not find out that I was missing some key tests until three days before finals and I failed two of my classes. I have since learned that I need to keep in constant communications with my teachers and make sure they work with me to complete my assignments and tests

Education Plans for Accomodations:

504 - Refers to Section 504 of the Rehabilitation Act of 1973, which prevents discrimination against someone with additional needs related to their disease, condition or health status, such as a person with CF.

IEP - Individualized Education Program - special accommodations for those with learning disabilities or other needs.

Contact your CF social worker for more information.

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PARENT TO PARENT

Attending the North American CF Conference: *A New Experience*

By Joe and Stacie Hallisy, Parents of a Child with CF

Our journey with CF started when the youngest of our three children, Rose, was born in 2016. She was diagnosed when she was 11 days old and the next day, we were at clinic learning "all things CF." That first year was a huge learning curve, and quite overwhelming. Over the years, though, we have gained some confidence and learned more about CF, which has been a big part of our life. Of course, every time we feel like we are on top of things and have CF figured out, it likes to throw us a curveball — constipation, a new cough, sudden resistance to doing treatments, or a different pharmacy we need to use for her medications.

This past November we were lucky enough to attend the North American Cystic Fibrosis Conference in Philadelphia. The theme of 2022's conference was "Back Together" as it was the first in-person conference since 2019. Everyone's excitement about being back in person was evident. We spent three days:

- Attending many different sessions led by physicians, respiratory therapists, social workers, researchers, nutritionists, nurses, and pharmacists. Some of the topics we learned about were adherence to daily CF therapies; racial disparities in healthcare; reviews of recent literature in newborn screening, lung transplant, palliative care and phage therapy; the impact of highly effective modulators on reproductive health & pancreatic disease; home spirometry; and managing headaches in people with CF.
- Exploring the hundreds of posters presented in the exhibit hall, focusing on everything from nutrition and treatments to modulators and CF-related diabetes.
- Attending plenary sessions (the big daily presentation that everyone attended).
- Browsing the vendors in the exhibit hall. We chatted with PARI Respiratory Equipment about nebulizer cups and tubing, tried out a few different vest systems to see how they felt, grabbed some stickers from RespirTech® for our daughter to decorate her machine with, and even picked up some cheesesteak recommendations from a Philly native.
- Spending time getting to know other CF parents and University of Michigan MOTT CF clinic staff.
- Hearing diverse outlooks on CF from individuals with CF, CF parents, and medical professionals from all around the world.
- Being amazed at all the different studies that are currently ongoing. We were encouraged at hearing all the great ways Trikafta ® has been beneficial for people with CF, and also how researchers are focused on finding therapies targeting those who are not currently able to benefit from Trikafta.

The best part of the conference was that our daughter Rose happened to turn 6 while we were there (Rose and her siblings spent a few fun-filled days with their grandparents while we were away). During one of the plenary sessions, we received a notification from the Michigan Medicine portal that her pharmacist had started the approval process for Rose to start Trikafta! She has been on Trikafta now for two months. The results have been positive so far after the first few weeks of her getting used to the new medication.

Many of these sessions were recorded and posted on the CF Foundation's YouTube channel on a playlist labeled "NACFC 2022." They are definitely worth browsing and choosing a few to listen to!

We came away from the conference feeling energized, better able to advocate for our daughter, more knowledgeable about the resources that are available to help us, and more confident in our understanding of the disease. It was truly inspiring to be surrounded by so many experts dedicated to finding a cure. We plan on attending future North American Cystic Fibrosis conferences (2023 is in Phoenix November 2-4, and 2024 is in Boston September 26-28) and hope that we will be able to meet some of you there as well!

TEAM UPDATES

Adult CF Program Patient Advisory Board Update

By Katie Hall, LMSW, Adult CF Program Coordinator

The Adult Patient Advisory Board has had an active year. We welcomed several new members and said goodbye as members rotated off the board. Our group has been active with helping bring issues to the forefront, always letting us know when it is time for updated communications regarding COVID-19. One initiative is the addition of "The Care Forward Connection," a quarterly newsletter sent through the patient portal. The Advisory Board wanted an opportunity to send out important information in one place. We will continue to evolve this, and if you have any topics you would like to see included, please send directly to Katie Hall: aultkath@med.umich.edu. Additionally, if you are not on the patient portal, this is another great reason to join! You'll be able to receive this and many other updates from the Adult CF Center. Let us know at your next clinic appointment if you would like to join or visit www.myUofMhealth.org to sign up!

As always, we are looking for new voices to join our Advisory Board. If you would like to learn more, please contact Katie directly.

Pediatric Family Advisory Board Update

By Catherine Enochs, RN, Pediatric CF Program Coordinator

The year 2022 brought about many great discussions with our Pediatric CF Family Advisory Board. We brought on new members and said goodbye to others. Our board worked diligently to revamp our new patient education materials, spending hours combing through content and wording. We successfully completed our first video on our educational video series, with more to come in 2023. We'll be looking to add more members in 2023, so stay on the lookout for a call for applications! Our Parent Partner and Advisory Board Member, Brandi Morgan, was nominated for an Evan Newport JOY award for excellence as a U-M volunteer. We also said farewell to Rebekah Raines, who moved on to other opportunities. Rebekah served as a Parent Partner and Advisory Board member with great dedication and skill. We're looking forward to planning our next project!

Video: https://healthblog.uofmhealth.org/childrens-health/how-to-help-kids-swallow-pills-7-easy-steps

New Education Materials (FAQ, clinic, admissions, travel tips): https://www.mottchildren.org/conditions-treatments/cystic-fibrosis/resources

GREAT STRIDES®
CYSTIC FIBROSIS FOUNDATION

5/6
Grand Rapids, MI

5/7
Detroit Zoo, MI

5/13
Ann Arbor, MI

5/20
Auburn Hills, MI
Montrose, MI

5/21
Toledo, OH
Mt. Pleasant, MI
Grand Haven, MI

CFF Great Strides!

The Cystic Fibrosis Foundation – Michigan Chapter holds several fundraising events including Metro Detroit's Finest, Purple Tie Ball, Detroit's Breath of Life Fall Ball, and Great Strides walks throughout Michigan and northwest Ohio. 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.

Advocacy

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.

Adult CF Program Quality Improvement (QI) Updates

Katie Hall, LMSW, Adult CF Program Coordinator

The Adult QI team has been active in several different initiatives through the CF Foundation and at our center. We have had a primary focus on improving our CF transplant referral process and educating all our patients on lung transplantation. This is an evolving process, and we are constantly revamping the program as we identify areas we can improve, or which are not working.

Our QI group is beginning to work on standardizing the IV antibiotics at our center. After reviewing literature, we have found that standardizing the process will be a helpful tool and will allow more patient involvement in the decision-making process. Soon, we will be sending out more communication on what this will look like, but this is the next project we are working on.

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TEAM UPDATES

The Pediatric Program Quality Improvement Projects – What Are We Working On?

When it comes to quality improvement, the Pediatric CF Program has an engaged and experienced team. Since 2020, we have been one of 36 CF programs that participate in the CF Learning Network, a quality improvement collaborative to develop and implement best practices for CF care through multicenter projects and collaboration. These are a few of our current and recent projects that will improve the quality of care we provide to people with CF and their families:

1. CF R.I.S.E. – We have a new initiative to improve the education we provide using the CF R.I.S.E. (Responsibility.Independence.Self-Care.Education) program for transition to prepare people with CF for adulthood. We invited Cincinnati Children's Hospital to collaborate with us to see how multiple CF centers can work together to find more solutions and better ways to optimize the use of this outstanding transition readiness program.

2. New patient education – With the help of our Parent Advisory Board, we've been evaluating our new patient education materials and revamping them to better fit what parents and families need. Be on the lookout for new materials! We'll be sharing widely as we create a great resource for families of our littlest CF warriors.

3. CF-Related Diabetes (CFRD) – Led by the Endocrinology department, Dr. Said has been evaluating CFRD screening trends in the past to help us improve screening efforts of CFRD in the future. Our CF team is also working on helping families and patients remember to complete screening through an oral glucose tolerance test starting at age 10.

4. Food Insecurity – Through team efforts and CF Foundation resources, we've adjusted how we screen for food insecurity to ensure we capture all patients and families. Using QI processes, we have taken a few steps, starting with attaching a screening questionnaire to the dietitian's visit check-in on the portal, in addition to verbal screenings when the portal is not completed. Of course, screening is only helpful if resources are provided, so the dietitians and social workers have worked together to provide individualized resources to those that screen positive.

5. Reducing Costs – Workup for CF after a newborn screen can become costly if your child does not produce sweat and we must perform full genetic sequencing. With the U-M Molecular Genetics Laboratory, patient financial counselors, and the state of Michigan's Newborn Screening Program, our center has been working on identifying patients who could benefit from a financial supplementation opportunity through Children's Special Healthcare Services. These communications have aided many families this year, with more to benefit in 2023.

6. Demographics – As we look internally at our care and practices for people of all races and ethnicities in the center, with the support and leadership of the CF Learning Network, we will be confirming race and ethnicity with each patient and family. To identify areas of need related to care of marginalized or minority populations, we must be able to compare our data on outcomes like weight, lung function, and prescription use across different races and ethnicities. Your honesty and understanding is appreciated as we work to highlight future areas of improvement.

Save the Date!

CF Night

The evening of April 26, 2023

In-person at the Kensington Hotel in Ann Arbor

Be on the lookout for an invite and RSVP info!

Faculty Introduction

Tara Havens, MD has officially joined the Pediatric CF Clinic as a new faculty member and assistant professor of pediatrics. She joins our team with experience in CF care and interests in a variety of pulmonary diseases.

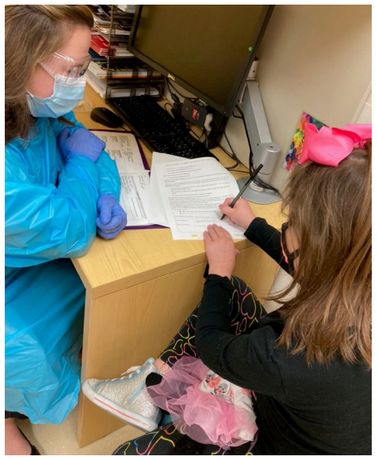
Quality Improvement vs Research: *What's the Difference?*

By Amy Filbrun, MD, Associate Director, Pediatric CF Program

At our CF Center, you may hear a lot about both research studies and quality improvement (QI) initiatives. Have you ever wondered what the difference is between these two activities? Research and QI are both very important ways to advance treatment and care of people with CF. Often they may seem quite similar, but there are some important differences.

Let's start with some definitions. "Research" is defined as a process of systematic inquiry that entails collection of data and analysis of that information in a way that is designed to develop or contribute to generalizable knowledge. Human subjects research is research involving a human subject, which is defined as "a living individual about whom an investigator conducting research: obtains information or biospecimens through intervention or interaction with the individual, and uses, studies, or analyzes the information or biospecimens; or obtains, uses, studies, analyzes, or generates identifiable private information or identifiable biospecimens." (Common Rule 45CFR46)

Quality Improvement is defined as "systematic, data-guided activities designed to bring about immediate positive changes in the delivery of health care in particular settings." (Hastings Report)



Annabelle providing assent, which is an agreement to participate in research for children of specific ages, done along with parental consent

When we consider these definitions, both involve the use of data in a systematic, or organized, methodical way. However, research is usually done in a way that will be generalizable to the world outside our center. It is often not related to current care, and is looking to test a hypothesis, or answer a very specific question. QI, on the other hand, is designed to implement knowledge or assess a program or process based on established norms or standards. In this sense, it is often the goal or endpoint that differs between research and QI. In research, the goal is to answer a specific question, such as "does drug A improve lung function in people with CF." The goal in QI is to improve a program or process, such as improving BMI in patients followed in our pediatric CF Center, to reach a target goal of BMI greater than the 50th percentile.

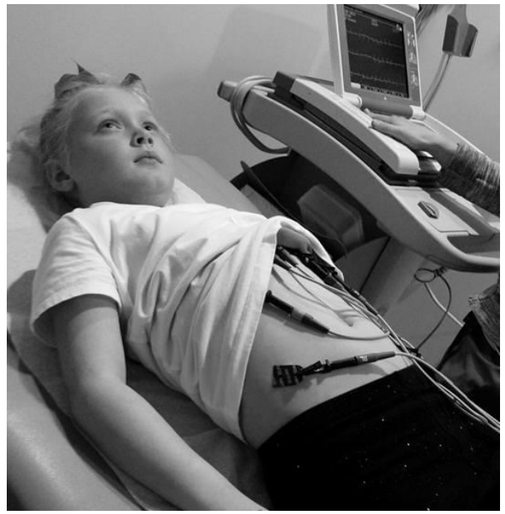
To achieve the research goal above, a randomized placebo controlled clinical trial is generally the best approach. Study participants are assigned by chance to receive either a placebo (no active medicine) or the study drug for a period of time, and they will undergo various measures to assess both safety and effectiveness of the study drug compared to the placebo. Often in these types of studies, participants are asked to accept some risk to participate. They are asked to provide informed consent, where all the possible risks and benefits, study procedures, etc., are explained to them before any activities take place. The research will follow a strict protocol that will not change for the entire study. Analysis is not completed until all participants at all sites have completed the study. Results often take some time to be analyzed and released. Sharing of results is generally expected from research projects.

The conduct of a QI project looks quite different. The team members for the center will often meet and brainstorm about the barriers to achieving the specified goal (e.g., improving BMI), and will then decide on a strategy to overcome that barrier and implement it immediately in clinic. Results of that change will be reviewed quickly—sometimes even after just a single clinic—and the team will decide if they need to adapt/change the strategy or adopt it. They will make changes and reassess again, adding new interventions as they are ready, to continuously be reassessing. These assessments are often referred to as plan-do-study-act (PDSA) cycles. The goal is to make frequent adjustments in order to keep what is working and move on from what is not. Often these cycles involve a process change in clinic that is very specific to the working of the clinic itself. Other times, the changes may be more generalizable to other centers that run similar clinics. Data is analyzed very quickly in a QI setting. QI projects do not usually put patients at risk, and consent is not obtained from patients, as the project really focuses on ways to improve the program or process. Often a patient, parent, or family member will be a part of the QI team, which really helps to understand how process changes may be perceived or may affect a patient and family. QI results, although perhaps not fully generalizable, are still helpful to share widely, and publication of results is encouraged.

The table pictured provides some additional comparisons between research and QI. Hopefully this has given you a better understanding of the differences between QI and research, and why both might be important to improving the lives of people with CF. Please feel free to ask us more about research and QI when you come to clinic visits.

CYSTIC FIBROSIS CENTER NEWS AND NOTES | VOL. XXXVI | SPRING 2023

CLINICIAN'S CORNER



Gracelyn (left) and sister Annabelle (right) having tests done for research.

	Human Subjects Research	Quality Improvement
Purpose	designed to develop or contribute to generalizable knowledge	designed to implement knowledge, assess a process or program as judged by established/accepted standards
Starting Point	knowledge-seeking is independent of routine care and intended to answer a question or test a hypothesis	knowledge-seeking is integral to ongoing management system for delivering health care
Design	follows a rigid protocol that remains unchanged throughout the research	adaptive, iterative design
Benefits	might or might not benefit current subjects; intended to benefit future patients	directly benefits a process, system or program; might or might not benefit patients
Risks	may put subjects at risk	does not increase risk to patients, with exception of possible patients' privacy or confidentialty of data
Participant Obligation	no obligation of individuals to participate	responsibility to participate as component of care
Endpoint	answer a research question	improve a program, process or system
Analysis	statistically prove or disprove hypothesis	compare program, process or system to established standards
Adoption of Results	little urgency to disseminate results quickly	results rapidly adopted into local care delivery
Publication/ Presentation	investigator obliged to share results	QI practitioners encouraged to share systematic reporting of insights

Table from https://www.research.chop.edu/services/what-needs-to-be-reviewed-by-the-irb#collapse-accordion-29860-4, adapted from the Hastings Report.

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