Cook Children's Cystic Fibrosis Newsletter

→ Special Edition

SPECIAL EDITION NEWSLETTER: NACFC 2015

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The 29th Annual North American CF Conference Kicks Off in Phoenix

The 29th annual North American Cystic Fibrosis Conference (NACFC) in Phoenix was a gathering of over 4000 researchers and healthcare professionals. The NACFC is the largest collaborative forum of its kind. We were all there to share and learn with the goal of improving the lives of everyone affected by Cystic Fibrosis. It was a great conference!

Last year, for the first time, Cook's CF Center brought you some of the highlights of the conference in a new way. We received positive feedback so we decided to repeat it. Many of the staff who attended the conference have provided a short summary of a session they attended. We hope you find this helpful and would love to continue getting your feedback about receiving the conference information this way.

CYSTIC FIBROSIS FOUNDATION

Attendees from 42 different countries have come together to share the latest breakthroughs in CF care, treatment and drug development. Sponsored by the Cystic Fibrosis Foundation, NACFC marks an international effort to conquer CF and combat the daily challenge of living with the disease.

From October 8 through October 10, 2015, NACFC featured three plenary presentations, a live one-on-one webcast and a variety of sessions addressing topics such as:

- Personalized medicine as a powerful tool for addressing rare mutations in cystic fibrosis.
- The integration of mental health as a critical component of specialized CF care, including the introduction of new screening and treatment guidelines for depression and anxiety.
- Meeting the challenge of increasing participation of people with CF in clinical research as unprecedented numbers of clinical trials are underway worldwide.

As the largest CF-specific scientific conference, key sessions will be available to the CF community through online streaming at: https://arc.nacfconference.org/cff/live/14

You will have to register, but there is no fee involved.

Enjoy our Special Edition Newsletter!

The Future of Personalized Medicine

John P. Clancy, MD, professor and director of CF Clinical and Translational Research at Cincinnati Children's Hospital and Medical Center

Personalized medicine -- also called precision medicine -- has received a lot of attention in the past year, including a mention by President Obama during the annual State of the Union address in January. But what does it really mean for people with CF? In the opening plenary session at NACFC, John P. Clancy, M.D., professor and director of CF Clinical and Translational Research at Cincinnati Children's Hospital Medical Center, discussed just that.

The idea of personalized medicine is nothing new. It is practiced at CF care centers every day. Based on their specific needs, individuals with CF are prescribed medications and therapies, such as antibiotics, pancreatic enzymes and airway clearance therapies. But more and more, personalized medicine is beginning to have a new meaning.

Clinical trials of ivacaftor (Kalydeco™) in people with G551D and other rare mutations clearly showed that drugs that target the malfunctioning CFTR protein at the cell surface have the potential to greatly improve health outcomes, including increased FEV_1 and decreased exacerbations. Major clinical trials of the ivacaftor/lumacaftor combination (Orkambi™) also showed significant improvements in people with two copies of the F508del mutation, with the drug helping the CFTR protein get to the cell surface and function properly.

However, we have also learned that drugs targeting CFTR protein can result in a range of responses, with some individuals showing significant clinical improvement while others have little to no response.

The range of responses highlights the need to find ways to determine who will benefit from a specific therapy. Dr. Clancy explained how we've already started to do this, and where we can go from here.

Researchers have developed ways of creating so-called model systems, essentially cell samples taken from the nose or other area of the body of an individual with CF. The hope is that doctors will be able to take a cell sample from an individual with CF, expose it to different drug compounds and then decide which drug would benefit that person the most.

These model systems are still being tested, but they represent just how customized care could become once a broader range of drugs becomes available. Dr. Clancy emphasized that once therapies are prescribed, it is important to monitor their effectiveness to ensure they are delivering the best results for the individual. It is also important to remember that CF is a multi-organ disease, and the effectiveness of treatments in parts of the body besides the respiratory system needs to be evaluated to understand their true impact.

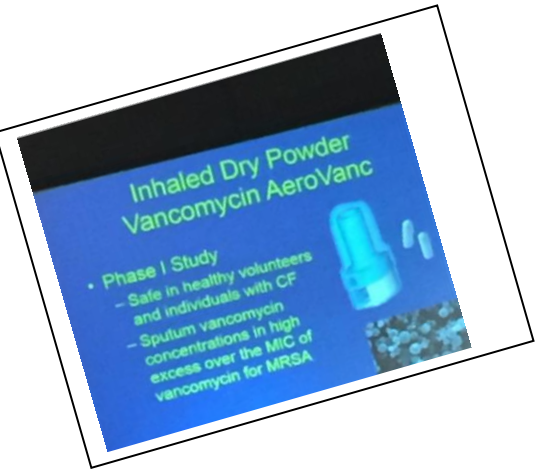
With the recent development and approval of the first drugs that target the basic defect in CF, an abnormal CFTR protein, the idea of personalized medicine means understanding an individual's underlying genetic problem and treating it directly.

In the coming years, we hope that doctors will have an arsenal of new therapies at their disposal to help provide the best care and treatment options for individuals with CF so that they can live healthy and fulfilling lives. In this new era, doctors will be able to use information about a person, including his or her specific CF mutations, lung function and bacterial infections, to select from a number of therapies to determine the best treatment options.

It is encouraging to see how the progress of drugs that target the underlying cause of CF has changed the lives of so many living with the disease. Walking out of this plenary, I am even more excited to think about the potential that these therapies and others in development have for improving the lives of individuals with CF in the near future. Personalized medicine at its finest

- RELIZORB -

RELIZORB is a digestive enzyme cartridge that contains the enzyme lipase and is used with enteral feeds to help break down fat in the formula. The cartridge that contains the lipase enzymes connects to the enteral tube feeding and as the formula comes down, it passes through the cartridge, and the fat will then be broken down into the absorbable form, fatty acids and monoglycerides. Each single use RELIZORB cartridge may be used for up to 500mL of formula and the rate must be in the range of 24-120mL per hour. RELIZORB breaks down 90-95% of fat into their absorbable form for most formulas tested and is compatible with both polymeric and semi-elemental formulas. The RELIZORB is currently FDA approved for adults and will hopefully be available to pediatric patients in the summer of 2016. The max amount that patients will pay for the cartridges will be $25.00 per month and the company will work with your insurance to get coverage for you. -Rachel Hamik, RD



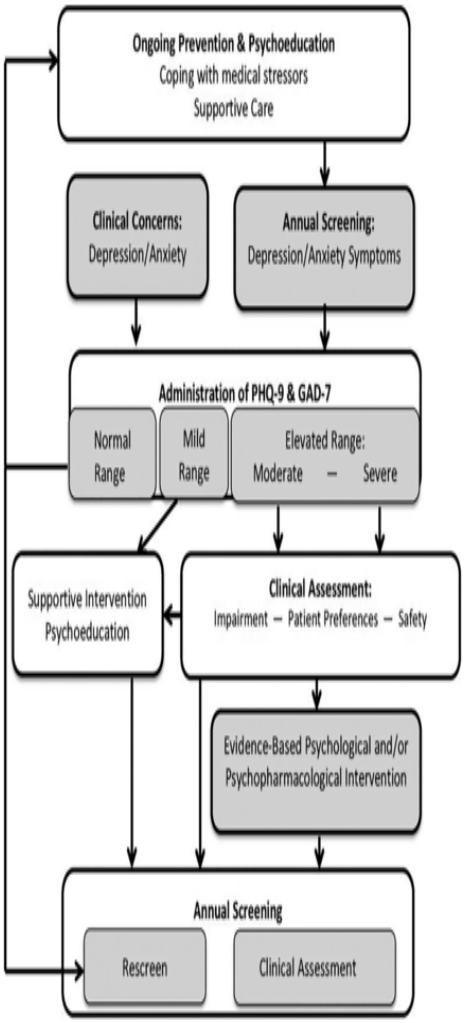
AEROVANC:

Let's get rid of that bug!

Deanna Pinckney, RRT

There is a new drug developing for MRSA related bronchopneumonia. It is currently in Phase 2 of the clinical trial. The drug is called Aerovanc and is an inhaled, dry powder form of Vancomycin. Vancomycin is currently the antibiotic of choice for CF patients, who have persistent MRSA lung infections. The only option for delivery, right now, is I.V. administration. This device is a capsule-based plastic device. The capsules are supplied in aluminum foil packages. The medication is delivered by placing one of the capsules in the well of the inhaler. The patient then pierces the capsule and inhales rapidly and deeply through the device. The hope is Aerovanc can deliver the medication directly to site of infection, reduce side effects and improve efficacy.

Figure 1: A flexible, stepped-care model for assessing and treating depression and anxiety.

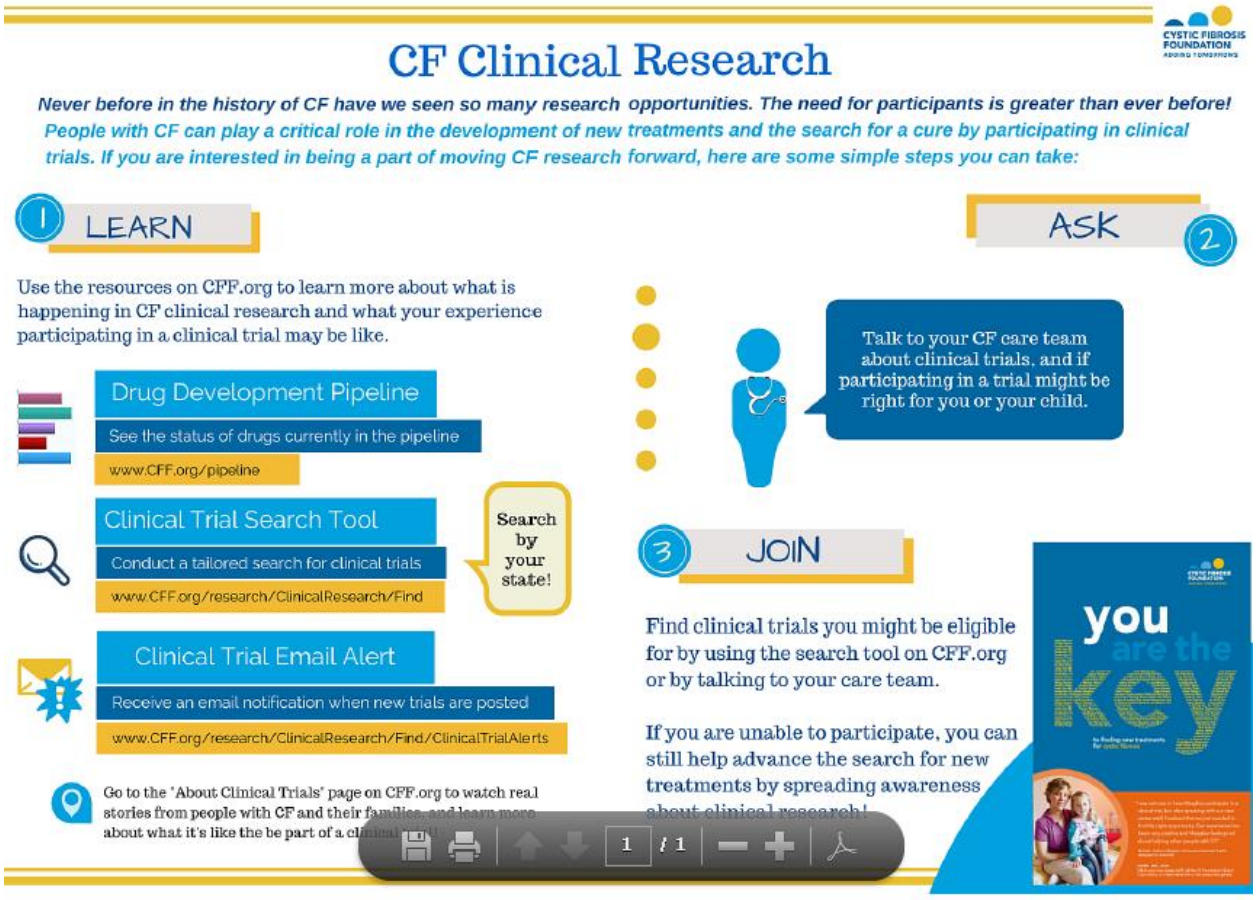


There's No Health Without Mental Health

One of the biggest themes for the North American Cystic Fibrosis Conference was the importance of mental health with CF care. The last plenary for NACFC 2015 was titled "*There is No Health Without Mental Health*." This plenary unveiled the new mental health guidelines by the CF Foundation and European Cystic Fibrosis Society. With the prevalence of anxiety and depression in individuals with CF and parent caregivers, it was important that a plan for providing the best care was prepared. International Committee on Mental Health in CF (ICMH) was formed to answer the question *how we can change clinical practice to improve mental health outcomes*. The committee recommended using the Patient Health Questionnaire 9 (PHQ-9), which includes an item to assess suicide risk, and Generalized Anxiety Disorder 7-item (GAD-7) Scale. The committee recommended that adolescents (age 12 years and older) with CF and adults with CF to be screened annually. They also wanted the PHQ-9 and GAD-7 to annually be offered to at least one primary caregiver of children with CF (ages 0–17 years). Along with the annual screening using the PHQ-9 and GAD-7, they can be administered due to concerns of anxiety or depression. The PHQ-9 and GAD-7 are brief questionnaires that are reliable for detecting psychological symptoms. It was recommended that caregivers that screen positive and have clinically significant symptoms are advised to follow up with their primary care physician or a mental health provider, outside of the CF team. The treatment plans for CF patients vary depending on clinical diagnosis as well as their scoring on the assessments, but not only on the assessment scores. The evidence-based psychological interventions that are recommended are cognitive behavioral therapy (CBT) and interpersonal therapy (IPT). CBT combines cognitive interventions with the principles of behavior modification. IPT is a short-term treatment that encourages patients to regain control of mood and functioning. Pharmacological treatments should be used in conjunction with psychological interventions. The new mental health guidelines, including screening, diagnosis, and intervention, will hopefully improve the quality of life and health of individuals with CF and their parent caregivers - Aditi Prabhakar

Cook Children's Clinical Trials

by: Heather Urbanek, RN, BS, CCRC



Cook Children's is participating in the following studies:

- Anthera study to evaluate Liprotamase, a pancreatic enzyme replacement therapy
- Nivalis SNO-6 study to evaluate a CFTR stabilizer in adult CF patients homozygous for the DeltaF508 mutation and being treated with Orkambi (accepting new patients!)
- CTX-4430-CF-201 study to evaluate an anti-inflammatory in adult CF patients (accepting new patients!)
- VX14 661 110 study to evaluate the VX-661 in combination with Ivacaftor in CF patients homozygous or heterozygous for the DeltaF508 mutation
- VX12 770 115 study to assess ocular safety of Ivacaftor
- CF Registry
- CFFC (fibrosing colonopathy) observational safety study to review the incidence of and risk factors for fibrosing colonopathy in US patients with cystic fibrosis treated with pancreatic enzymes
- EPIC Observational Study 11-15 years which will collect specimens to increase the biorepository
- VX15-371-101 study to evaluate safety and efficacy of VX-371 in CF patients homozygous for the DeltaF508 mutation and being treated with Orkambi (in start-up phase, accepting new patients!)
- And more new studies every day!

Heather Urbanek, RN, BS, CCRC

Clinical Research Coordinator

Cook Children's Medical Center

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THE IMPORTANCE OF PHYSICAL THERAPY AND MANAGING CYSTIC FIBROSIS

As people with CF are living longer, fuller lives, we are starting to see more of the side effects of this lifelong disease. Teens and adults with CF can develop postural deformities, pain, stress incontinence, and limitations in range of motion and strength. When the constant demand to breathe takes extra physical effort and energy expenditure, typical motor patterns other bodily functions take second place. This chronic pattern can take a large toll on joints and normal movement, and this is where the physical therapy department can help. PTs are skilled in helping individuals correct muscle imbalances and restore optimal alignment and movement. This year's NACFC offered several hands-on sessions to provide tools to evaluate and treat these conditions. We want to make sure people not only survive into adulthood and beyond, but thrive.

Allison Ewing, PT, DPT
Physical Therapist
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CookChildren's Medical Center

The Cystic Fibrosis Center at Cook Children's Medical Center is one of more than 115 accredited CF centers throughout the nation. These centers are accredited by the Cystic Fibrosis Foundation, a non-profit organization founded in 1955 dedicated to funding research to find a cure for CF and improving the quality of life for people with the disease

Pediatric CF Center Staff:

Center Physicians:

- James Cunningham, MD– Co-Center Director (Nurse: Stacy),
- Nancy Dambro, MD– Co-Center Director (Nurse: Kara & Karen),
- Maynard Dyson, MD (Nurse: Stacy),
- Sami Hadeed, MD (Nurse: Sharon),
- John Pfaff, MD (Nurse: Jessica),
- Karen Schultz, MD (Nurse: Paulette),
- Erinn Newman, DO (Nurse: Lisa)
- Anunya Hiranrattana, MD (Nurse: Juliann)

Adult CF Center Staff:

Center Physicians:

- John Burk, MD– Adult Center Director,
- Jack Gilbey, MD
- Stuart McDonald, MD
- Randall Rosenblatt, MD
- Cyndy Roger, ACNP-BC (Nurse: Candace, adult nurse and newborn screening)

CF Coordinators:

- Janet Garbarz, Carrie Stradley 682-885-6299

Dietitians:

- Cara Dennert, Rachel Hamik, Esther Giezendanner 682-885-7496

Respiratory Therapists:

- Deanna Pinckney, Alex Rasmussen, Crystal Thompson, Cindy Corne, Shonda Thompson 682-885-4189

Child Life Specialist:

- Elise Roswell, CCLS 682-885-4892

Social Services

- Aditi Prabhakar 682-885-3991

Research Nurse:

- Heather Urbanek (Heather Leach, research associate) 682-885-1244

Clinical Therapist:

- Mariah Snapp

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