

COOK CHILDREN'S CYSTIC FIBROSIS CENTER

NEWS

VOLUME VII

WINTER 2011

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.

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We're Moving...Open House

Cook Children's Pulmonary Center will still be providing the same quality of service and care to our CF patients, but the center will be moving to a different location!

The new building will be open for business March 26, 2012. The CF clinic will be located on the 2nd floor of the Dodson Specialty Clinic. Please use the new parking garage located at 7th Ave. and Pennsylvania.



Open House for the new CF clinic is scheduled for March 20, 2012 from 4-8 pm. Come on in and check it out!!

Please let us know if you have any questions regarding the location or parking garage for the new clinic. Be sure to consult with the CF staff regarding whether or not your next appointment will be scheduled at the old or new clinic site.



Some exciting new features of the new CF clinic include:

- More treatment rooms
- Centralized registration on the first floor
- No toys or books in the treatments room to assist with infection control
- Gel and masks will still be provided



Cook CF Patient Spotlight:

Madison Ramsey



1. Tell us a little about yourself...

** Hi, I'm Madison, and I am 14 years old. I am in 8th grade and attend Hunt Middle School in Frisco, Texas. I have a sister named Shelby, who is 17 years old and she is always supportive of me in my long journey with my illness. She's a great sister, and when I'm in the hospital, she always decorates my room with posters and my name in big letters over my bed.

2. When were you diagnosed with CF?

** I was diagnosed when I was 4 months old.

3. What are some of your favorite hobbies/activities?

** I really love my iMac, and I enjoy editing videos and creating projects on it. I aspire to be a video editor. I love to read, hang out with my friends, and play with my dog.



I am currently in YAC-PAC, the Cook Children's Youth Advisory Council. I really want to improve the hospital and make it a better place for kids.

I'm also a FAAB VolunTEEN at the Frisco Public Library. FAAB stands for Frisco Action Advisory Board and we meet twice a semester to work on projects to better Frisco. As a VolunTEEN, I volunteered this past summer at the Frisco Public Library doing jobs such as helping people check out books, restocking shelves, and helping with special programs such as story time.

I'm in a teen Anime Club at our public library. We meet once a month to share our interest in anime books and Japanese culture.

I am the leader for our Cystic Fibrosis Foundation's Great Strides walk team. I invite walkers to walk with us, have a web page on the CFF walk site and produced a video of what it is like to have cystic fibrosis. Last year, we raised \$6500 for a cure.

I also volunteer at the Frisco Family Services Food Pantry. I stock groceries on the shelves as well as help "shoppers" determine how many of each item they may have.

4. What are some of your favorite foods

*** My favorite foods are mashed potatoes and gravy, fruit, and chocolate. I have a big sweet tooth. I also crave salty foods, like fries, because it's good for CF kids to get

enough salt.

5. How do you balance life with CF?

*** I balance life with CF by doing everything I can to stay healthy, doing all my medications and treatments. Sometimes I don't even think about it, because it's so routine with me. I just try to be a normal teenager! I also go half days to school when I'm doing home IV's. Kids at school ask me about my port when it is accessed and I tell them all about it. I don't let CF stop me from traveling or sleeping over at a friend's house. I just take all my meds with me. I have been on a cruise, parasailed, snorkeled, rode horses on a dude ranch, gone to the beach, gone to Disney, etc. I have my passport and want to use my Make A Wish this summer to go to Tokyo.

6. Tell us something unique about yourself?

*** Something unique about me is I have a very good memory, and I like to write stories a lot.

7. What are your future goals?

*** My future goals are to be a successful video editor, to be a novelist, because I love writing books, and to own three dogs because I love dogs.

8. What's a piece of advice that you might give to someone else with CF?

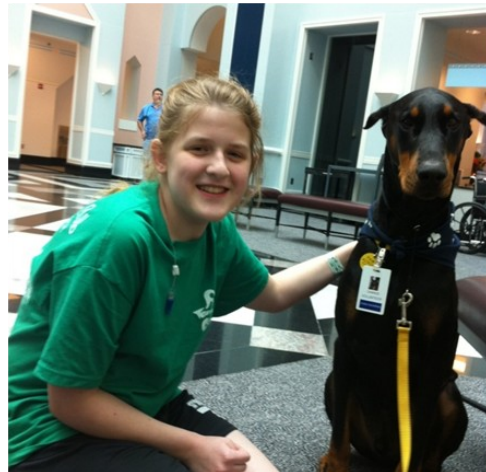
*** A piece of advice I'd give to someone else with CF is to always do your medications and treatments, because even if they are really annoying to do, you'll be thankful you did later down in the road. Also, salt salt, salt! Salt is very good for you, so pound it, baby! If you don't like PICC lines, get a port because it is much easier and less painful getting IV's and blood taken. Also, I carry my own enzymes at school which is a lot easier.

9. How long have you been a patient at Cook CF Clinic?

*** I have been a patient at Cook since 2004, for 8 years. So far, I love Cook! Best hospital ever!

10. What do you like best about Cook?

***I really don't have a favorite! I love everything about Cook, the gift shop, the cafeteria, but probably my most favorite is the therapy dogs that come to visit. I love seeing them every time I'm in the hospital. They remind me of my own dog I have at home, Rose.



New Online Library Helps CF Patients Navigate the Challenges of Health Care Coverage

Cystic fibrosis patients and their families can now turn to a new, helpful collection of online resources when faced with health care coverage and reimbursement challenges. At the North American Cystic Fibrosis Conference in 2011, the CF Foundation announced the launch of the **Patient Assistance Resource Library**, its latest program dedicated to providing the CF community with the information needed to gain and maintain coverage.

A range of useful information is now publicly available in the new online Library:

- Sample letters of medical necessity and prior authorization
- How-to guides for navigating common insurance obstacles
- Templates for insurance appeals and exception
- CF care guidelines
- Medical journal articles
- White papers for medical professionals

The Library's contents are drawn from Foundation educational material as well as information from health care providers and industry partners.

"The CF Foundation is pleased to provide another tool to help patients and their families navigate the health care maze," said Robert J. Beall, Ph.D., president and CEO of the Foundation. "We are dedicated to helping people with CF gain better access to the treatments they need to maintain and improve their health."



[Register NOW for Great Strides 2012](#)

Where to Stride:

Saturday May 19: Granbury– City Park, Nocona– Downtown Nocona, Weatherford-Holland Lake Park

Sunday, May 20: FW/Arlington– Six Flags

Saturday, September 15: Abilene– Red Bud Park

** Contact our local CF Chapter for any more information on how you can get involved, ftworth-ne-texas@cff.org

Is Your Compressor Doing What You Need It to Do?

Prescribed inhaled medication for CF treats the basic airway problems: inflammation and infection. Delivery of these medications to the site of the disease in the airway is the purpose of aerosol therapy.

While more drugs are being developed for the treatment of CF, the potential for more time spent taking these medications may also increase. It is the responsibility of the CF health care team to ensure that the equipment that patients are using for the delivery of inhaled medications is efficient and keep the time burden to a minimum.

A compressor is a small, electric air device that produces pressurized airflow. It is paired with a nebulizer medicine cup that holds the medication to be aerosolized. The nebulizer/compressor combination, along with the medication characteristics, is what determines the length of time that each medication takes to nebulize. The order of inhaled therapies and length of time that each nebulized medicine should take to be delivered is:

- Bronchodilator (albuterol, Xopenex)- Delivery time: 10-12 min.
- Hypertonic Saline- Delivery time: 10-12 min.
- Mucolytic (Pulmozyme)- Delivery time: 8-10 min.
- Antibiotic (TOBI)- Delivery time: 15-20 min.



If your medications are taking much longer than the suggested times, you may need to talk to your CF respiratory therapist.

Some issues to consider:

1. How old is the compressor that you are using? Most private and state insurance companies will replace a unit every 3-5 years.
2. Has the filter on the compressor been changed in the past 6 months? Every compressor has a filter that helps prevent dust particles from getting in to the compressor motor. The medical equipment company that supplied the machine will have replacement filters.
3. Is the amount of nebulized medications taken on a daily basis too much burden for a small air compressor? You may want to discuss purchasing a larger compressor.
4. Are the nebulizer medicine cups clean and disinfected, and have they been replaced in the past 6 months? Proper cleaning and disinfection of the medication cups are essential for fast and efficient medication delivery. Most home nebulizer cups need to be replaced every 6 months.
5. Which nebulizer cup should be used with which medications? Review which medications are being delivered in a Pari, Sidestream, or other nebulizer cup.

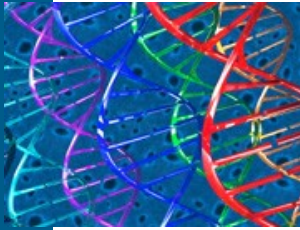
**Be sure to talk with your RT if you have any questions



Targeting Mutations that Cause Cystic Fibrosis

New drugs in development are NOW available for patients to receive treatments that specifically repair the genetic mutations responsible for their cystic fibrosis (see pg 8). Understanding which type of mutation a patient has could pinpoint which new drugs could work for them.

More than 1,000 mutations in the CF gene cause the disease. Researchers classify these mutations into six types. Using these classes, scientists can better understand the disease and are developing therapies to treat it.



What Do the Mutation Classes Mean?

Not every case of cystic fibrosis is the same. A person's mutation determines what goes wrong on a cellular level, and which therapies may work to correct it.

The CF gene makes a protein called the Cystic Fibrosis Transmembrane conductance Regulator (CFTR). The protein is a chloride channel. Chloride, a charged particle in cells, flows out of the cell through the center of the channel and water follows behind it. The water keeps mucus thin so it can move out of the lungs.

In healthy people, CFTR is located on the surface of cells lining the lungs and other organs. People with CF have little or no CFTR, depending on what class their mutation falls into.

What Are the Different Mutation Classes?

In three mutation classes, insufficient CFTR is made — Classes I, II and V. Class I mutations are nonsense mutations. Because of missing genetic information, these mutations stop cells from making the complete CFTR protein. In Class II mutations, CFTR folds incorrectly. The malformed protein cannot move from the inside of the cell to the surface, where it is needed. The most common CF mutation, Delta F508, is a Class II mutation. Class V mutations decrease the amount of functioning CFTR that makes it to the cell surface.

In the other mutation classes — III, IV and VI — CFTR is produced and delivered to the cell surface, but the mutations keep it from working properly once it is there. Patients with these mutations sometimes have less severe CF symptoms because there is some functioning CFTR on the cell surface.

The “engine” of CFTR is dysfunctional in Class III mutations. The protein does not respond to chemical signals that tell it to work, explains Steven Rowe, M.D., assistant professor of medicine at the University of Alabama.

In Class IV mutations, not enough chloride moves through the opening of the chloride channel. Class VI mutations cause the protein to pull away from the cell surface too quickly, creating a shortage of CFTR.

Why Do Mutations Matter?

Identification of mutation classes gives researchers a better understanding of how certain drugs, currently in clinical trials, may help specific CF patients.

CFTR-targeted therapies could work at the cellular level, addressing the cause of CF rather than treating symptoms.

“We hope these classes will allow us to develop treatments that target the basic defect and restore health in people with CF — possibly in ways we never imagined,” said Rowe.

A Quick Reference to CF Mutations

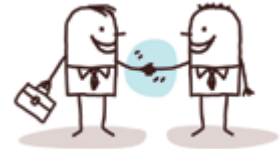
Cystic fibrosis mutations fall into six basic categories or classes. Here is a quick guide to the mutation classes and what they mean.

Mutation	What's gone wrong
Class I	Stop signal in CF gene occurs too soon; no CFTR is made
Class II	CFTR is misfolded keeping it from reaching the right place; affects 80 percent of CF patients
Class III	CFTR is made and in right place, but does not function normally
Class IV	Opening in CFTR is faulty
Class V	CFTR is made in smaller than normal quantities
Class VI	CFTR degrades too fast; not enough protein is present

The Winter Season: Germs are Ready to Move In

Germs (pathogens), like bacteria and viruses that cause lung infection, can spread between people in many ways. These are known as routes of transmission. The 3 main ways are (1) by contact, (2) in a droplet or (3) through tiny remains of droplets floating in air (airborne).

The most common way germs spread is by contact. This is also called direct or indirect contact transmission. Direct contact is when germs spread by bodies touching, such as through shaking hands, hugging or kissing. Viruses that cause common colds, respiratory syncytial virus (RSV) and CF specific germs like *Pseudomonas aeruginosa* (*Pseudomonas*) and *Burkholderia cepacia* complex (*B. cepacia*) are spread this way.



Indirect contact involves touching something with germs on it, like touching a doorknob or sharing a cup. Germs spread to you when you touch something with germs on it and then touch your eyes, nose or mouth. When a person talks, sings, coughs, sneezes or laughs, droplets are made. These tiny drops of liquid may have germs inside. The droplets with germs can land in the eyes, noses or mouths of others. This is how germs can be spread by droplet transmission. These drops can travel 3 to 6 feet through the air before they fall to the ground. The flu (influenza) and whooping cough (pertussis) are spread this way. Some germs travel through the air on specks of dust or particles made when a person talks, sings, sneezes, coughs or laughs. These germs can float in the air for a long time. They can be carried a long way by air currents. Illness occurs when people breathe in the germs floating in air. Tuberculosis (TB), measles and chicken pox are some of the germs spread by airborne transmission.

Prevent Infections

- Clean your hands with soap/water, hand sanitizer
- Keep shots up to date
- Use and throw away tissues
- Clean and disinfect nebulizers
- Avoid sick people
- Do not share eating utensils or cups

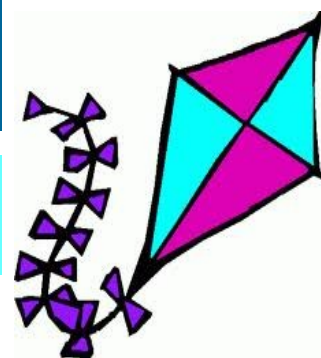
When to Clean Hands

- At a doctor's office, waiting room or lab.
- After you cough, sneeze and blow your nose.
- After using automatic teller machines (ATMs), handrails, elevator buttons, public telephones, grocery carts and other people's pens.
- At shared play places and gymnasiums.

Hope for the Future...

FDA Approves Kalydeco (VX-770)- First Drug that Targets the Underlying Cause of Cystic Fibrosis

The Cystic Fibrosis Foundation applauds the Food and Drug Administration's approval of Kalydeco™ (ivacaftor; previously known as VX-770), a major advance in the search for a cure for cystic fibrosis.



The drug was developed by Vertex Pharmaceuticals Inc., with scientific, clinical and significant funding support from the Cystic Fibrosis Foundation.

The FDA approved Kalydeco (kuh-LYE-deh-koh) for a segment of the CF population, those ages 6 and older with the G551D mutation of cystic fibrosis. The drug is taken in pill form.

"Today marks an important milestone in our journey to find a cure for cystic fibrosis," said Robert J. Beall, Ph.D., president and CEO of the Cystic Fibrosis Foundation. "Kalydeco addresses the underlying cause of CF, and the science behind the drug has opened exciting new doors to research and development that may eventually lead to additional therapies that will benefit more people living with CF."

The G551D mutation is present in roughly 4 percent of the CF patient population in the United States. In people with this mutation, a defective protein caused by CF moves to its proper place at the surface of the cell but does not function correctly. Instead, the defective protein acts like a locked gate, preventing the proper flow of salt and fluid in and out of the cell.

Kalydeco helps unlock that gate and restore the function of the defective protein. The drug dramatically improves lung function, lowers sweat chloride levels and helps patients gain weight — all key clinical indicators of CF.

The CF Foundation played a significant role in the research and development of this groundbreaking new drug. "The unique and mutually beneficial partnership that led to the approval of Kalydeco serves as a great model for what companies and patient groups can achieve if they collaborate on drug development," said FDA Commissioner Margaret A. Hamburg, M.D., in a news release issued by the FDA.

While Kalydeco represents a breakthrough for patients with the G551D mutation, it also represents hope for all people with CF, according to Preston W. Campbell, III, M.D., the CF Foundation's executive vice president for medical affairs. "Our organization's mission is to find a cure for cystic fibrosis, and we won't stop our work until we reach our goal for all people living with the disease."

The vast majority of individuals with CF in the United States – nearly 90 percent – have a different mutation, called Delta F508.

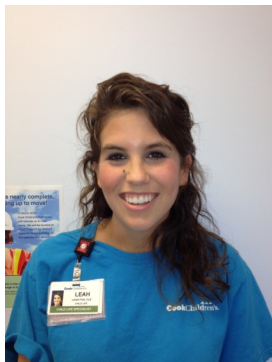
Developing therapies for patients with the Delta F508 mutation is among the current research priorities of the CF Foundation. Progress toward this goal includes an ongoing Phase 2 clinical trial of Kalydeco in combination with another potential therapy, VX-809, in people with the Delta F508 mutation. Results from the first part of this Phase 2 trial were positive. The second part of the study is now under way.

In addition, the CF Foundation has significantly expanded its research investments with other leading pharmaceutical companies, including Genzyme and Pfizer, to accelerate the discovery and development of new drugs that will help more CF patients.

Referenced from: www.cff.org

A big WELCOME to our newest Cook Children's CF Staff

Leah Fox, Child Life Specialist



Leah has been working in the Pulmonary clinic since September. Prior to that, she was completing her internship at Cook Children's on the inpatient floors. Leah is a graduate of the University of Alabama and a diehard fan! She is looking forward to getting to know the families at Cook Children's and to another Alabama football season!



Cindy Corne, Respiratory Therapist



Cindy has been a respiratory therapist for 30 years. This is her first time to work in a Children's Hospital. She has worked at CCMC for about 4 years now. She really enjoys her job at Cook and "loves" working here. She attended RT school at Midwestern State University. Cindy is married and has 3 children, as well as 1 cat and 2 dogs.

CookChildren's

Cook Children's Medical Center Cystic Fibrosis Center

Pulmonary Services
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Ft. Worth, Texas 76104-2724
Phone: 682-885-6299
Fax: 682-885-1090
Email: www.cookchildrens.org

Making CF stand for "Cure Found"

Pediatric CF Center Staff:

Center Physicians: **James Cunningham, MD**– Center Director (Nurse: Stacy), **Nancy Dambro, MD**– Center Director (Nurse: Charity & Karen), **Maynard Dyson, MD** (Nurse: Stacy), **Sami Hadeed, MD** (Nurse: Sharon), **John Pfaff, MD** (Nurse: Tiffany), **Karen Schultz, MD** (Nurse: Paulette)

Adult CF Center Staff:

Center Physicians: **John Burk, MD**– Adult Center Director, **Steve Davis, MD**, **Stuart McDonald, MD**, **Cyndy Roger, ACNP-BC** (Nurse: Sofia, adult nurse and newborn screening)

CF Coordinators:

Janet Garbarz, Carrie Stradley 682-885-6299 (#6)

Dietitian:

Staci Brummett, Cristina Puga,
Julie DeBois 682-885-7496

Respiratory Therapists:

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

Child Life Specialist:

Leah Fox 682-885-4892

Social Services

Jamie Cole 682-885-2863

After hours and on weekends/holidays, call 682-885-4000 and ask the operator to page the pulmonologist on call.

Cook's Corner: Recipe for Success

Power Oatmeal

Ingredients

1 cup cooked oatmeal
1/2 teaspoon cinnamon
2 tablespoon ground flax seed
1 cup plain Greek yogurt
1/2 cup granola
1 cup blueberries, raspberries and strawberries
1/4 cup almonds
2 tablespoons local honey to drizzle

Directions:

1. Stir cinnamon and flax seed into prepared oats.
2. Divide between two bowls.
3. Divide almonds and top oats.
4. Add 1/4 of the berries to each bowl.
5. Layer Greek yogurt on top of berries.
6. Drizzle honey on yogurt.
7. Add divided Granola then finish off with remaining berries.

2 servings
570 calories per serving
Fat: 27 g
Sodium: 43 mg
Protein: 19 g
Calcium: 196 mg

