COOK CHILDREN'S CYSTIC FIBROSIS CENTER

FORT WORTH, TX.

Cook Children's Cystic Fibrosis NEWS

VOLUME II SUMMER 2010

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.

INSIDE THIS ISSUE:

Jump Into Summer

Summer Fun in the Sun With Cystic Fibrosis

If you're itching to get out and indulge in some summer fun in the sun, don't let cystic fibrosis stop you! With a little advance preparation there's no reason why you can't participate in many of the summer activities you enjoy. Follow these guidelines to ensure you stay safe as you take the plunge into a fun-filled summer.

Consult Your Doctor

Exercise is an important part of cystic fibrosis management and will undoubtedly be included in your treatment plan. However, you should not participate in any activities that you haven't discussed with your doctor first. Your cystic fibrosis specialists are familiar with the unique details of your physical condition and are best prepared to recommend activities that are right for you. Be sure to let them in on your summer plans.

Stay Hydrated

High temperatures can cause dehydration very quickly, especially when combined with physical activity. For people with cystic fibrosis, dehydration is especially dangerous because the water loss increases the thickness of mucus.

Avoid dehydration by drinking plenty of fluids before you become thirsty. A good rule of thumb is to drink 6-12 ounces of water or other non-caffeinated beverage for every 30 minutes spent exercising or in the sun.

Replace Salt and Calories

Heat and physical activity will cause you to burn extra calories and lose salt through sweat. Check with your CF dietitian, but you will probably need to eat foods to replace what you lose.

If your nutritionist agrees, be sure to pack plenty of salty snacks such as:

- Sports drinks
- Nuts
- Pretzels
- Potato chips
- Trail mix

Keep it Cool

Before you head out this summer, check with your pharmacist to get storage instructions for your medications. Some common cystic fibrosis medications must be refrigerated, others should be stored at room temperature. Never leave your medications in the car, and keep them away from direct sunlight.

Avoid Allergens and Pollutants

Make sure that you know what's in the air you will be breathing. Avoid areas known to have high levels of smog or pollution because these things contain chemical irritants that can trigger breathing problems. The Environmental Protection Agency maintains a searchable database of air quality information that's a great tool to check out the areas you plan to visit.

It's also a good idea to be aware of any environmental allergies that you may have and avoid areas that are likely to contain them. If you can't avoid allergens, you may want to ask your doctor if an antihistamine is appropriate for you.

Keep Medicine and Supplies at Hand

If you are traveling by air, be sure to keep your medicines and necessary supplies in your carry on bag. Airlines do lose luggage sometimes, and lost bags can take days or weeks to recover. Never pack anything in your check-in baggage that you can't live without for that period of time.

Some things you will want to keep with you:

- Medicines
- Nutrition supplements
- Your doctor's contact information
- Medical equipment

Stick to Your Routine

It's easy to get side tracked when you're away from home, but it is important to stay as close to your usual daily routine as possible. Continue to take your medications at prescribed times, and plan your activities around respiratory treatments.

Having a serious or life-threatening medical condition can make it even harder to cope with already-difficult realities of being a teenager. Supporting a brother or sister who has an illness can be daunting as well. With this in mind, Starlight Children's Foundation created *Starbright World*®, an online social network exclusively for sick teens and their siblings.

Starbright World is an online social network where teens (ages 13 to 20) who have serious medical conditions, and siblings of seriously ill teens, can connect with each other via moderated chat rooms, games, bulletin boards, videos, and more. *Starbright World* is a virtual hangout where teens can build on existing friendships or create new ones, from home or from the hospital.

Connect Many teens living with chronic and life-threatening illnesses are unable to leave the hospital or their homes, which often results in feelings of isolation and getting left behind by friends. On *Starbright World,* teens find that they are not alone and establish meaningful relationships with others their own age who are struggling with the same issues. They have multiple ways to connect with each other, such as the moderated chat room, bulletin boards, polls, ecards and more.

Games *Starbright World* boasts an exciting collection of both interactive (Battleship, Connect Four, Mankala) and single-player (Sandwich Shop, Gem Swap) games. To keeps things lively, the players with the highest scores are posted directly on the site, allowing members to comment on the game activity.

Videos Last but definitely not least is the post-your-own video section of the site where members can share their creative genius with their friends about their diseases, lives, or anything they want to showcase.

To learn more about this program, email your question to www.starlight.org/starbrightworld

Living An Abundant Life

Spotlight on Carol Shepherd a 52 year mother with CF.

My name is Carol Shepherd, I am 52 years old and was diagnosed with Cystic Fibrosis at age 5. At the time of my diagnosis, my parents were informed that I would not live past age 13. My mother took me for monthly doctor visits for many years and did her best to take care of me and my three siblings without CF. My mother was very nutrition-minded in her meal planning and made sure we had many healthy foods as well as some fun foods. I will always consider that an asset to my good health.

During my early years, most of my CF issues were digestion problems. After being diagnosed with CF I was put on enzymes, but the early enzymes were not as effective as current enzymes are. I also had sinus problems and surgeries. I went to school, played, took dance lessons, rode bikes, ran, and tried to live a normal life. My family moved frequently until my father retired from the military and settled in Austin, Texas when I was 15. As a teenager, I started having lung-related hospitalizations during the spring allergy seasons. I dated and had boyfriends. I met Robert, now my husband, at age 16 and we started dating a year later. I started gainful employment at age 15 and then left for college at age 17. I made it through 4 years at what is now Texas State University San Marcos without too much more than very lengthy colds and oral antibiotics. I graduated with a B.S. degree in Family Ecology and a minor in Social Work. I had done some volunteer work in college that was required for my social work classes.

I knew that I wanted to be a social worker, marry and have children, and someday do volunteer work again. At 21 I was on my own, employed as a Texas A&M agricultural extension agent. About a year and a half later I went to Fort Worth and I took a position as a supervisor/social worker. Robert and I were married and he continued graduate school full time at Texas Christian University. After living in Fort Worth awhile I eventually found the CF clinic (which was only once a month in an old house). We had one doctor and about 30 patients going to clinic then. During a hospital stay, I was told women with CF often could not get pregnant and it could be life threatening for me to have a baby. Later I found out adoption agencies would not let me adopt a child because of CF. My husband and I were heart-broken. Thankfully, many of the things I was told back then have changed now!

After three and a half years of marriage my husband and I were blessed with twins! I was the first person with CF ever known to give birth to twins where mother and both children survived! (It was published in a medical journal.) That was when my life became challenging! I knew I had to do my treatments and take care of myself the best I could so that my sons would have a mom. The doctors, nurses, friends, family, and my husband and I found ways to take care of my health, so I could be there to raise our sons. I felt like I had three full-time jobs: two boys and me! I no longer worked outside the home. My husband finished school and with his Ph.D. in Physics, took an aerospace job and taught classes at night. The babies grew and as they did, I did all the things many mothers do, except sometimes I was attached to an IV medication. I volunteered at the boys' school, went to school field trips, field days, parties, and soccer games, and watched my sons play in band. Only once I missed the first day of school because of a hospitalization, and my mother came from Austin to substitute for me. I taught both boys driver's education and drove with them to and from work until they had a license and car available. Then I helped them find scholarship applications and visit colleges. We attended church regularly and I taught Bible study at times. Just thinking back on all the treatments and all the activities is exhausting now! (I did not even mention cooking, cleaning house, doing laundry, ironing, taking care of a dog, shopping, and so on). My faith in God and His plan for me was always what kept me going.

I have a strong faith, but I also believe I have to do the things that are in my power to do, such as go to doctor appointments, take medications, and do treatments as instructed. If those with CF want to live a "normal" life, they must do all the things needed to keep healthy. The hospitalizations are not fun, but I use that time as a chance to recharge physically and spiritually. I do have a port in my arm for IV use and think I should have had it put in sooner! The port has been in 19 years now. There have been many sinus surgeries, intestinal blockages, and a diagnosis of CF-related diabetes, along with countless lung infections, various other surgeries, and a few non-CF health issues. I have had somewhere near 100 hospitalizations. My first hospital stay was in a ward full of other kids, all of us in cribs even though I was 5. That time and another I had no T.V. to watch and my parents were never allowed to stay with me in any hospital I stayed at as a child. Eventually I was in Cook Children's Medical Center, a little piece of heaven! Now I go to a hospital where appropriate adult care is provided.

In recent years I have had more severe lung infections, and 2 years in a row a pulmonologist told me I could have 3 months to 1 year to live. My main goal at that time was to be at my son's wedding. I made it to his wedding and overcame that infection after a long period of treatment from a really great infectious disease doctor. From that experience I learned that infectious does not mean contagious. Today I am well, with no help from the pollen in the air giving me allergy trouble! Next month I could be in the hospital, but for now I will keep on doing what I do and when the next hospitalization comes along I will deal with that. Each thing that comes along is one more thing to deal with. It never becomes routine or normal, but I have become accustomed to frequent hospitalizations.

There are many things I have gained from having CF. I have a very strong relationship with God. I have a great appreciation for what prayer can accomplish! I have more time during illness to really study my Bible. I have many friendships with others who have CF and/or parents, and I have met some of the most wonderful healthcare providers. I gained personal depth and maturity at a young age, as well as a strong relationship with my mother. I have had many opportunities to encourage others. On the fun side, I pretty much get to eat anything I want!

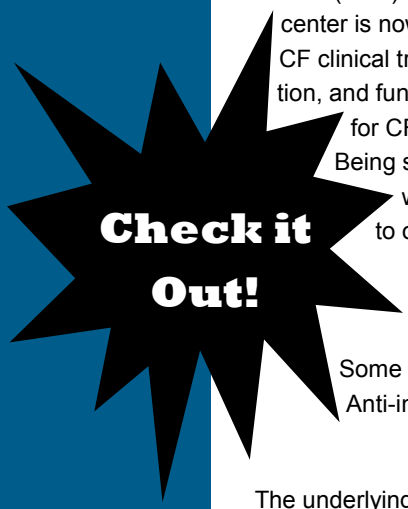
My husband, Robert, and I have been married for 29 years. Our sons, John and James, are fine young men, 25 years old and graduates of Texas A&M. James is married and works at NASA where he trains astronauts. John is a civil engineer in Fort Worth. I am still living my dream of doing meaningful volunteer work by intermittently working for the CF Foundation, the CF Family Advisory Council, and weekly as a counselor at a Woman's Center in Fort Worth. I have also participated with drug studies for CF-related drugs. I spend as much time as possible with my mother who has been with me all along my CF journey (if not physically, always prayerfully). It is only because of the Lord's help and my husband's support that I have been able to fulfill my purpose in life. Our hope for the future is to become grandparents, but my sons have to help with that one! It is always my great privilege to talk to others with CF or their family members. I am listed in the Fort Worth phone directory or you may check with the CF coordinators for information on how to reach me.

Cook Children's Role In CF Research

Our CF center's role in research

In January 2009 the Cook Children's CF Center was chosen as a Therapeutic Development Center (**TDC**) by the Cystic Fibrosis Foundation Therapeutics, Inc. (**CFFT**). This means that our center is now part of a network of nearly 80 CF centers nationwide specializing in performing CF clinical trials (research). The CFFT provides these centers with information, tools, education, and funding to facilitate the safe, efficient, and coordinated evaluation of new treatments for CF.

Being selected as a TDC is a competitive process. Our amazing clinic and staff along with our successful involvement with research projects over the years contributed to our center's selection.



What's new in CF research?

Some of the most active areas of research are CFTR modulation, Anti-inflammatory and Anti-infective medications, and therapies that restore airway surface liquid.

CFTR modulators

The underlying cause of cystic fibrosis is a defective protein called CFTR (cystic fibrosis transmembrane conductance regulator). This protein makes a channel (like a gate or passageway) through the cell's surface. In the cells that line the lungs and other organs, CFTR allows chloride and sodium (salt) to move properly in and out of cells. Defective CFTR moves salt and water poorly, if at all, as indicated by increased amounts of chloride in the sweat. CFTR modulators are medications designed to correct the function of defective channels.

Three CFTR modulators are currently under study: Ataluren, Vx-770 and Vx-809. All three medications are designed to be taken **by mouth** every day.

Ataluren (PTC124) aims to treat CF patients who have what is known as a nonsense (or stop codon) mutation. Nonsense mutations interfere with the cells construction of CFTR and cause CFTR to be too short and non-functioning. Nonsense mutations are the cause of CF in about 10% of CF patients.

Vx-770 is a CFTR "potentiator" designed to help CFTR located at the cell surface to function better.

Vx-809 is a CFTR "corrector" designed to move defective CFTR from inside the cell to its proper place at the cell surface.

Vx-770 for the G551D mutation

Most of the research with Vx-770 has been with CF patients with the G551D mutation. The G551D mutation results in CFTR that gets to the cell's surface but does not work properly. Early results from these studies show improvements in lung function and chloride movement through the CFTR channel in these patients.

Vx- 770 for the Delta F508 mutation

Our CF center is one of several participating in a clinical trial studying whether Vx-770 helps patients with two copies of the Delta F508 mutation. Delta F508, the most common CF mutation, makes a defective CFTR that the cell destroys instead of moving it to its place at the cell surface.

Results from this study will not be available until all patients have completed their participation in the study and the information collected has been analyzed.

Vx-809 for the delta F508 mutation

An initial, short term study looked at the safety VX-809 given to CF patients with the delta F508 mutation. These patients showed a decrease in sweat chloride levels while taking Vx-809. This suggests that this drug helped the defective CFTR get to the cell surface and work correctly. More studies with Vx-809 are anticipated.

VX-809 plus Vx-770 for the Delta F508 mutation

Researchers believe that patients with the Delta F508 mutation may get the best results from using Vx-809 and Vx-770 together. The Vx-809 will move the defective CFTR to the cell surface where Vx-770 will improve its function.
The first study to look at VX-809 and VX-770 together in CF patients is currently in the planning stages.

Improving hydration of the airways

Research is ongoing with medications such as Denufosol and Bronchitol™ that improve airway surface liquid. In CF, changes in salt transport within cells results in dry, thick and sticky mucus. These therapies aim to thin the mucus so it can be cleared out of the lungs more easily. Hypertonic saline is an example of this type of therapy that is already available to patients.

Anti-inflammatory

Drugs in this category decrease inflammation in the lungs of CF patients, which should help decrease long-term damage to lung tissue. Ibuprofen is one example of this type of therapy that is already used with CF patients but many others are in earlier stages of study.

Anti-infective

Several new drugs are being studied to learn their effectiveness in destroying bacteria (germs) that cause short-term and long-term lung infections. TOBI , Azithromycin, and recently approved Cayston belong to this category and are already available to patients.
Our center participated in the tobramycin inhaled solution (TOBI) and tobramycin inhaled powder (TIP) studies. While TOBI was approved and has been in use for several years, results from the TIP studies have not yet been released.

Information about clinical trials

Proving that potential new treatments are safe and effective often takes a long time. It can be several years between the start of a clinical trial and the time a new therapy is available to patients. However, knowing the therapies currently being studied provides a picture of what treatments may be available to patients in the future.

With more than 30 therapies currently being developed for CF it would be difficult for a single CF center to participate in all the clinical trials. To make sure patients have the opportunity to participate in trials important to them, TDCs work together and refer patients to each other's centers for specific research studies.

To learn more about current CF research, talk to:

- Your CF doctor or nurse
- The CF coordinators, Janet Garbarz and Carrie Stradley
- The Research Nurse, Sara Scott

If we don't know the answer we know where to look for it.

You can also find a great deal of information on the following web sites:

- www.cff.org/research
- www.nhlbi.nih.gov/childrenandclinicalstudies
- www.clinicaltrials.gov/ct/info/resources
- www.fda.gov/oashi/clinicaltrials

Live Web Cast- Thurs, Aug. 19

Partnering for Care:

Help your CF Care Center Help You Thrive*

Watch the Webcast Live! Part 1 of a 5 part Series

Thursday, August 19, 2010 at 7 p.m. Central

Hosted by: Johns HopkinsHospital & featuring:

Michael Boyle, M.D., F.C.C.P.

Director of the Adult CF Program

Peter Mogayzel, Jr., M.D., Ph.D.

Director of the CF Center and Pediatric Lung Transplantation

Join us for the first chapter of the *Partnering for Care* Webcast Series to learn how to partner with your doctors to help your CF care center help you thrive!

Drs. Mogayzel and Boyle will address questions about the following topics:

• How to partner with your child to reach your goals in managing CF

• How to partner with your care center in the transition from pediatric to adult care and the varying aspects and concerns with the change

• How to partner with your adult care center to successfully manage CF in your daily life

• How to partner with your care center when considering lung transplantation

Pre-registration begins **July 8th** on the CF Foundation's Website, www.cff.org or www.cfWebcast.org.

Questions can be submitted during registration and the live broadcast!

Cook Children's Medical Center
Cystic Fibrosis Center

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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: Karen and Charity), **Maynard Dyson, MD** (Nurse: Stacy), **Sami Hadeed, MD** (Nurse: Sharon), **James Pfaff, MD** (Nurse: Chrystal), **John Saito, MD** (Nurse: Liz), **Karen Schultz, MD** (Nurse: Paulette)

Adult CF Center Staff:

Center Physicians: **John Burk, MD– Adult Center Director, Steve Davis, MD, Stuart McDonald, MD**

CF Coordinators:	
Janet Garbarz, Carrie Stradley	682-885-6299 (#6)
Dietitians:	
Staci Brummett, Christina Gonzalez	682-885-7496
Respiratory Therapists:	
Deanna Pinckney, Alex Rasmussen, Ruthie Oney, Crystal Thompson	682-885-4189
Child Life Specialist:	
Amy Kaufhold	682-885-4892
Social Services	
Jamie Cole	682-885-3991

After hours and on weekends/holidays, call 682-885-4000

The Cook's Corner: Recipe for Success

Enjoy this tasty treat to celebrate the summer!

Peach Cooler

1 cup peaches

1 cup whole milk

2 drops vanilla flavoring

1 cup vanilla ice cream

Combine all ingredients except ice cream into a blender. Process at liquefy. Add ice cream and blend until smooth.

Makes one serving:

560 calories.

**You can use any type of fruit for this high calorie snack