CYSTIC FIBROSIS News Wire

VOLUME TWO - NUMBER TWO -Spring 2010

Welcome to the latest edition of the Children's Hospital Boston CF Center Newswire. We hope that you continue to find the CF Newswire a valuable source of information. In this issue, we have included updates on many exciting clinical, educational, and research activities ongoing at the CHB CF Center. May is also National Cystic Fibrosis Awareness Month, and many of you participated in your local Great Strides Walk. We hope you and your families were able to find inspiration in these gatherings. Happy Spring!

Gregory Sawicki MD
Associate Director, CF Center

NEWS ABOUT PANCREATIC ENZYMES

April 28, 2010 was the deadline set by the US Food and Drug Administration (FDA) for approval of all pancreatic enzyme replacement therapies. This approval process started in 2004, and over the past several years, clinical trials were completed in patients with CF in order to meet the FDA requirement. The goal of this process was to standardize the manufacturing process for enzymes in order to ensure consistency from batch to batch. This effort was strongly supported by the CF Foundation.

As of the April deadline, 3 enzyme products had received approval by the FDA: Creon, Zenpep, and Pancreaze. There are other products that are currently available to patients that may still receive FDA approval after the deadline has passed. If you or your child are currently prescribed an enzyme product that has not yet been approved, you will need to discuss a plan with your physician. For more information, please speak with your individual CF physician. Information can also be found on the CF Foundation website at http://www.cff.org/treatments/Therapies/Nutrition/Enzymes/

CF FAMILY EDUCATION CLASS

BY AMY FLYNN

Brendan was diagnosed with Cystic Fibrosis at 2 weeks of age by our Obstetrician by testing the cord blood. He is the youngest of 3 boys and the only child with CF. Our initial reaction to the diagnosis was shock, sadness and fear of the unknown. When we received the invitation to the "new diagnosis seminar" my first reaction was not to attend. I was unsure of the setting and did not want to hear depressing stories of what the future may hold for my child. My mother convinced me to go for Brendan. I am so glad that I did. We met wonderful families that were in the same situation. We learned about what to expect and not to expect and most of all- we had a lot of laughs. I enjoyed every aspect of the morning and was disappointed to have to leave early to attend my oldest son's hockey game. The group of families had my same worries, fear and concerns. I also learned I was not alone. I hope to meet these families again in the future and wish them the best of luck! Thank you to Dr Sawicki, Monica Ulles, NP and Kate Barnico, RN for putting together an informative, but relaxing, educational seminar.

Dr. Sawicki, Brendan, Tim and Amy Flynn

A new inhaled antibiotic called CAYSTON was recently approved for the treatment of Pseudomonas in CF. If your physician has prescribed this medication, it is supplied with an Altera nebulizer. The new Altera nebulizer has only been studied with the medication cayston and should not be used with other inhaled medication.

My Story

Jennifer Denoncour

If anyone looked inside my purse they'd probably say it resembles a pharmacy. You have no idea how glad I was to see big, bulky purses back in style. There are times when I've had enough of all this extra stuff. After all, none of it is even for me. But how could I not lug it around? Being the wife of a person with Cystic Fibrosis doesn't come without some major responsibilities and toting around a bunch of meds is just one of them.

My husband is my best friend and whatever he goes through, I go through. When he can't sleep at night, I can't sleep. When he's stuck in the hospital, I'm in the hospital. If he has to fast for lab work, I fast too. Bottoms up to a nice chalky protein drink, and make it a double. It's difficult encouraging and preparing high calorie/ high fat meals when it's everything I shouldn't be eating, but we're in this together and sometimes that means sacrificing for the other person.

There are times when I have to be the bad guy, speaking up at clinic when I don't think he's doing as well as he's claiming or pushing for him to workout when I know it's the last thing he feels like doing. I find myself waking him up to tell him he hasn't finished the last neb of the night and sometimes have to scold him when 3 weeks have gone by and he still hasn't called to schedule an appointment for clinic or with one of the other specialists. I hate being the bad guy, but somebody has got to do it. It is after all for his own good.

When I hear from people what a happy couple we are in spite of everything I tell them laughter truly is the best medicine. It's ok to poke fun, even if it's about something serious, because it gets us through it. I try not to live life stressing about what may or may not happen, because while focusing on the bad there is potential to miss out on some wonderful moments. It's hard to think about the future and make plans for down the road when no one knows just how long that road may be. I worry about the day when we become a one income family, but try not to let it get the best of me. I don't have to like what may or may not be in store for us, but it's extremely important to accept it.

CF has made our marriage stronger because it gives us something so many other couples don't have…a glimpse into the future. We appreciate each other so much more because we know we don't have an infinite amount of time together. We don't take each other for granted like some couples and try to always remember that the little things are just that…little. Life is meant to be lived and enjoyed and we're doing just that….CF and all.

KEEPING YOUR LUNGS HEALTHY

BY BRIDGET CHAMBERAS

On March 6th we attended Dr. Dorkin's presentation "Keeping your Lungs Healthy".

It was a great opportunity for my wife and I to have a Saturday morning "date", but more importantly it was very informative. We learned many things, including that the thick and excess mucus in the lungs of people with CF prevent the lungs from compressing fully when they exhale. Dr. Dorkin illustrated to us that this was like taking a deep breath and only being able to let out about half of the air taken in. This demonstration led into an explanation of the Pulmonary Function Test or PFT. This test not only measures how much air is inhaled or exhaled, but also the force with which it is done over a discrete time period.

The session was also very inspirational to us as well. At first we were intimidated by how well informed some of the parents were with the physiology and treatment options, but then realized that we all manage the stresses and emotion of CF in our lives differently. Some of us by being as informed as possible, some of us by diving into fundraising and others by raising awareness. The session also reminded us that it has been some time since we had been evaluated on how we administer PT. We used that reminder to schedule an appointment with our physical therapist for an evaluation.

All in all this was a great session - we look forward to the next one!

Bridget and Anthony Chamberas - Honored at the Cystic Fibrosis Foundation Annual dinner for Outstanding Volunteer Award 2009 Photo by Sheryl Lanzel

Lauren Bombardier essay entry winner Solvey scholarship 2009

My dream for the future is to ignore every stormy forecast and to embrace the beauty in the sun. When I do get caught in the rain, however, I will dance in that rain and come inside with wet hair and a better outlook on life. I plan to continue the attitude I have now to fight a potentially harder fight and to one day see a cure. I wish to continue to better myself every day because of the lessons I have learned from having cystic fibrosis: an appreciation for life, the importance of determination, and being prepared. I dream to continue to appreciate life but to be able to laugh until my belly hurts without stopping to cough. I dream of being determined and running a marathon without having to stop for breath. I dream of living past the expectations to see my retirement but I also know that I must be prepared for whatever obstacles this disease presents. Because of my positivity, I dream of being a survivor, and teaching others' to have a positive outlook on life. Thus, I dream of becoming an English teacher. I want to teach my students not only how to read and write but how to learn life lessons from stories. I want to teach them that every character has a struggle, and it is with this struggle that we become better characters. I want to show them that surviving adversity teaches us how to appreciate life and never to take anything for granted. I will be a living example of this. Maybe one day cystic fibrosis will be a thing of the past. Until then though, it is my goal to continue to take care of myself so I can survive the toughest fight of my life. And through this survival, I and every person I have touched will have learned to dance in the rain.

Josh Napolitano creative entry winner Solvay scholarship 2009 "While I breathe, I hope"

Of course, like anyone with an illness I would dream for a cure to get rid of my disease, but for now I live with it. My dream for the future is that people with CF would have the advantage of innovative advancements in medicine -- like I have had. Four years ago my brother and I were very sick after contracting an undiscovered genus of drug resistant mycobacterium. Subsequent medical journals say that our case was the first ever reported anywhere on earth. Our doctors were perplexed about what to do to eliminate this bug and spent two years researching a treatment. They called what they did "trailblazing" because the entire process was experimental. Eventually after several attempts we were able to combat it.

Getting rid of that bug gave me the opportunity to live. I have been healthy enough to excel at school, play on the school's Varsity tennis team (becoming captain this year) and achieve the rank of Eagle Scout. I look forward to studying astrophysics at college next year.

During this low point I faced my own mortality. I lost my innocence but I emerged from the experience a different person with a different perspective. Everyone should have a second chance. That experience in retrospect has not only given me an

SolvayCARES Scholarship — Applicants include U.S. citizens with CF who are enrolled or awaiting acceptance from an accredited institution in the fall. Awards are based on applicants' creativity, academic excellence, community involvement and ability to serve as a positive role model for the CF community.

nutrition

by Kristen Leavitt, RD, LDN

We are excited to announce the addition of a new dietitian at the CF Center. Her name is Ashley O'Brien and started May 3rd. You will start seeing her in our clinic in July. She will be part of the inpatient and outpatient CF team, and part of the lung transplant team. We are excited that she is joining our team!

I recently returned from a CF Nutrition and Social Workers Conference in Indianapolis, Indiana. We received updates from the CF Foundation, including data that confirms the importance of having a Body Mass Index (BMI) at the 50th percentile or greater. We learned a lot about feeding tubes and how beneficial they can be for people with CF who are struggling to get their BMI up to the 50th percentile. We also learned about lung transplantation. One of my favorite speakers was Tiffany Christensen, a woman with CF who has had 2 double lung transplants. She is an author and national speaker, and gave us all great insight into what it is like living with CF and how we can be better. I also enjoyed hearing from a panel of parents and children with CF who discussed their experience with feeding tubes and what they felt helped them.

The CF Foundation recommends that every patient meet with nutrition at least yearly or more often if your BMI is less than the 50th percentile. Please be sure to make an appointment to see nutrition the next time you are in for an exam if you have questions or concerns, or it has been a while since you have seen nutrition.

Color Your Calories!

I wanted to share some ideas with you on how to include more fruits and vegetables as part of your meals and snacks.

Plant foods, such as fruits and vegetables, contain compounds called phytochemicals. These compounds play a role in keeping your body healthy. Some of the well known phytochemicals are called antioxidants, such as beta-carotene. The best way to get these great compounds into your body is to eat fruits and vegetables every day. However, fruits and vegetables alone don't have a lot of calories in them. Below you will find some ideas on how you can add these fruits and vegetables to your diet without losing out on calories. The most colorful fruits and vegetables are the ones that have lots of these antioxidants in them, so think about all the colors you can add to your foods!

Color your Food with Fruit

	Breakfast, Lunch & Dinner	Snacks & Desserts	Beverages
Quick Tips…	Sprinkle raisins or dried berries on hot cereal. Eat cold cereal with sliced fresh fruit. Eat a blueberry muffin Add dried or fresh fruit to salads.	Eat peanut butter with apples or bananas. Top them with raisins or dried cranberries. Add fruits to ice cream and yogurt. Order fruit pie instead of cake.	Squeeze sliced lime or lemon into lemonade or iced tea. Add whole fruit to beverages Try strawberries in strawberry milk, or raspberries or strawberries in lemonade. Order fruit juice instead of soda.
Got a few minutes…	Top pancakes or waffles with fresh fruit in addition to syrup and butter. Add fresh fruit to yogurt.	Make ice cream sundaes with fresh fruit in addition to the chocolate sauce and sprinkles. Make a fruit cup of mangos and grapes.	Blend strawberries into a puree. Add to lemonade for homemade strawberry lemonade. Make a fruit smoothie (see recipe).
Now you're cooking…	Have a slice of mango-pineapple salsa with pork chops in addition to the gravy. Serve cranberry, orange relish with poultry.	Bake a cranberry-apple crisp. Try a kiwi, plum or strawberry tart. Make your own trail mix with raisins, dried cranberries, nuts and chocolate chips.	Blend fruit with milk and instant breakfast (see recipe). Make hot cranberry cider, add orange slices.

Share your favorite recipe at Cfevents@childrens.harvard.edu

Color your Food with Vegetables

	Lunch	Dinner	Snacks
Quick Tips…	Order sweet potato fries instead of regular french fries. Add roasted red pepper slices to sandwiches. Find them jarred, frozen or even roast your own. Order a side salad with dressing.	Top pizza with vegetables such as mushroom, red and green peppers and broccoli. Drizzle melted cheese on broccoli.	Enjoy salsa with corn chips. Serve raw vegetables with dip. Drink vegetable juice. Scoop peanut butter with carrots or celery
Got a few minutes…	Try creamy soups with color: cream of carrot, butternut squash or even pumpkin. Several brands are available in the grocery store.	Add garlic to vegetables; make garlic mashed potatoes. Bake sweet potatoes with olive oil, salt and pepper. Crown baked potatoes with cooked vegetables and cheese.	Try spinach artichoke dip (it comes frozen) or guacamole dip. Have a slice of vegetable pizza.
Now you're cooking…	Grill or sauté eggplant slices and add them to sandwiches. Use avocado slices on sandwiches or add chopped avocado, black beans, chopped red peppers and onions to regular macaroni and cheese for a healthier high-calorie tex mex dish.	Add cooked green beans, zucchini, asparagus, broccoli, or sautéed green leafy vegetables to cheesy quiches and casseroles. Use sundried tomatoes in creamy alfredo dishes.	Try carrot salad with raisins, apple and pineapple (see recipe). Eat a slick of carrot cake or zucchini bread (see recipe). Make a cheese & vegetable omelet. Try tomatoes, spinach, onions or peppers.

colorful recipes

Smoothie

1 cup 100% fruit juice
½ cup whole milk vanilla yogurt
½ cup strawberries
1 small ripe banana

Blend ingredients. Serves 2.

Instant Breakfast Shake

1 cup whole milk
1 envelope strawberry instant breakfast or scandi shake
½ cup strawberries
½ ripe banana

Blend ingredients. Serves 2.

Sweet Potato Muffins

Preheat oven to 400 degrees F.
One 29 oz can sweet potatoes, drained and mashed.
Two 7.5 oz packages corn muffin mix
¾ cup whole milk
2 large eggs
2 Tbsp sugar
1 Tbsp vanilla extract
1 tsp ground cinnamon
1 tsp ground nutmeg

Mix sweet potatoes, sugar, eggs vanilla and spices. Add corn muffin mix and milk. Stir until smooth. Grease muffin tins. Bake at 400 degrees for 20-25 minutes. Makes 12 muffins.

Carrot Raisin Salad

2 cups raisins
1.5 cups grated carrots
2 apples with skin, cored and cut into pieces
Two 8 oz cans crushed pineapple, drained
1 Tbsp lemon juice
½ cup mayonnaise

Combine all ingredients. Serves 9.

Wheat Germ Zucchini Bread

Preheat oven to 350 degrees F.
1 ½ cups sugar
2 cups all purpose flour
1 cup wheat germ
1 tsp baking soda
½ tsp baking powder
½ cup chopped nuts
1 cup applesauce
2 Tbsp canola oil
3 large eggs
2 cups grated zucchini
2 tsp vanilla

Combine sugar, flour, wheat germ, baking soda, baking powder and nuts in a medium bowl and set aside. In a large bowl, combine the applesauce, oil, eggs, zucchini and vanilla. Add dry ingredients and stir until just blended.

Grease and flour two 8x4 inch bread pans. Divide the batter between the two. Bake at 350 degrees for 45 minutes to 1 hour.

Makes 2 loaves of 12 slices each.

RESEARCH CFTR MODULATORS*

ERIN LEONE, MPH, CCRC

There has been increased interest in a type of promising therapy called CFTR Modulation. In CF, the faulty CF gene results in a problem in the production or expression of the CFTR protein channel, which causes an imbalance of salt (sodium) and water (chloride) on the surface of cells lining the lungs, gastrointestinal tract, and other organs. CFTR Modulators are investigational drugs that aim to correct the function of the CFTR protein channel, so that chloride and sodium move in and out of the cells properly. In this way, CFTR Modulators may be able to address the basic defect in CF. CFTR modulators are designed to target specific CFTR gene mutations.

Clinical trials of CFTR Modulators are necessary to determine whether these drugs are safe and effective in patients. It is important to remember that these studies are necessary to gather information about the therapies that will be reviewed by researchers, clinicians, and the U.S. Food and Drug Administration (FDA) to decide if they should be approved for widespread use. These research studies involve tests such as lung function tests (breathing tests), laboratory tests (blood tests), and sweat chloride tests (sweat tests), which provide data about the safety and effectiveness of the drug in patients. Although CFTR Modulators are very promising therapies, it usually takes several years for a treatment to move from clinical trials to patients.

The CF Center at Children's Hospital Boston is involved with several ongoing studies of CFTR Modulators sponsored by Vertex Pharmaceuticals and PTC Therapeutics. These studies are described below.

Vertex VX-770 is a CFTR potentiator for patients who have at least one copy of the G551D gene mutation. Patients with this gene mutation make the CFTR protein channel, but it does not function properly. A CFTR potentiator aims to increase movement of chloride through the faulty CFTR protein channel. In early small studies, VX-770 was shown to be safe for patients. Additionally, short-term treatment (14 or 28 days) with VX-770 in a small number of patients improved lung function tests and decreased sweat chloride levels. VX-770 is currently being evaluated in three Phase 3 studies called the ENDEAVOR Program. These studies are looking at the effects of VX-770 on a larger number of patients over a longer period of time (16, 24 or 48 weeks).

Vertex VX-809 is a CFTR corrector for patients who carry two copies of the F508del gene mutation. Patients with the F508del gene mutation make the CFTR protein, but it does not reach the surface of the cell. A CFTR corrector aims to move the CFTR protein channel from the inside of the cell to the surface of the cell. In early studies, VX-809 was found to be well-tolerated by patients. Additionally, some patients had decreased sweat chloride levels. The next study planned of VX-809 will look at using it in combination with VX-770 for patients who carry the F508del mutation.

PTC Ataluren (formally PTC124) is a CFTR modulator being tested for patients who carry a nonsense gene mutation (contains the letter X). A nonsense mutation interrupts the production of the CFTR protein. This modulator has the potential to allow a functioning CFTR protein to be made. In previous studies, Ataluren was shown to be generally well tolerated by patients. Additionally, atatluren treatment over two 14 day treatment cycles was associated with trends toward decreased cough frequency and improved lung function tests. Currently, a Phase 3 study of Ataluren is being conducted to determine the effects of the treatment on a larger number of patients over 48 weeks.

*Information for this article was gathered from www.cff.org, www.vrtx.com, and www.ptcbio.com

If you are interested in learning more about CF clinical research, please contact a member of our Clinical Research Team or visit www.cff.org

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patient family ADVISORY council

PFAC continues to meet monthly at the Children's Hospital Waltham location. We are always interested in having new members join our group. Our mission is to help empower patients and families to be active partners in their CF care. Together we are dedicated to getting the most comprehensive and compassionate healthcare for our patients. To achieve this we have delivered lectures, contributed to the Newswire, collaborated with the Center directors and brainstormed ideas to educate, inform, and improve the experience for our patients and families. We are currently working on lectures for the fall and to develop other avenues to provide information that are useful and accessible to all.

Do you have a question that has gone unanswered? Why not join us and explore the PFAC and help us deliver the answer for you and others who would like answers and information. The group meets the last Tuesday of the month at the Waltham location. Please email either elllbrown@yahoo.com or amorypjulian@comcast.net for more information.

FAMILY TO FAMILY MENTORING PROGRAM

Isabel Bailey SW, Judy Bond SW, Lynn Helfand SW

What do I say when friends ask "what is CF'? How do I juggle taking care of my daughter who has CF and my other 3 kids? How do I get a 504 school plan for my son? What do I need to pack as I plan for my own or my child's hospitalization? How do I handle my first job interview? How do I know whether it is the right time for my son to pursue lung transplantation?

These questions and musings arise every day for families and adult patients who are living with cystic fibrosis. Some answers to these questions may come out of discussing these issues with CF Center staff. But talking with others who have faced similar situations and have experienced the joys and challenges of raising a child with a chronic medical condition offers unique and specialized support and can be enormously validating and reassuring. Experienced parents love letting others know about their "hard-earned tips and tricks." Sharing this feeling of community can decrease a sense of isolation, increase understanding and confidence and can contribute to a family's quality of life.

Our CF Mentoring Program, launched a few months ago, was initiated to help families with identified questions connect with other parents who have had experience dealing with similar issues. This project has been collaboration between our Patient/Family Advisory Council, our CF Center staff, and the Center for Families. Volunteer mentors include parents of young children, adolescents, adults and one adult patient who have had diverse experiences living with CF. On a Saturday morning in January; ten potential mentors attended a training program facilitated by Center for Families staff. This established family to family program was adapted to our program and prepared patients/parents to provide one on one telephone support to other patients or family members who may have specific questions related to living with CF. This match is meant to be short term and consists of approximately 2 phone calls over a period of a few weeks. Mentors do not provide medical advice but rather help other patients and parents express their feelings and emotions, formulate their questions, and potentially connect them with resources that can address their concerns.

We are very excited about this new venture and are eager to hear from each of you who may want to be matched with a mentor. We urge you to talk either with your CF physician and/or your CF clinic social worker about your interest in connecting with a mentor (or in becoming a mentor). Judy Bond, one of the CF Center social workers, will contact you to talk about the program and will connect you with a mentor who most closely matches your request for support.

congratulations

Awarded the Frances Pew Hayes Family Foundation Hope In Action Award 2009 Presented To Audrey Clark For Efforts In Furthering The Mission Of The CFF

Photo: Audrey Clark and her mother Sandy GaffeyPhotos by Sheryl Lanzel

September 11, 2010 will mark the 5th Annual Ride in memory of Steven J. Vertuccio. The events is a 50 mile motorcycle ride for more information go to www.sjvride.com "Over the past four years we have raised about 40K, our goal for this year is10K" says Laura Vertuccio Steve's sister in-law.

Billy Sullivan, Rick Vertuccio and Laurie Vertuccio presented a check of $9.000 to the Cystic Fibrosis Center at Children's Hospital.

Dr. Donovan, Dr Sawicki, Dr. Dorkin and Kate Barnico, RN

CONTACT INFORMATION

Phone numbers:

Appointments: 617-355-1900 option #3

Nursing line: 617-355-7018

Prescription line: 617-355-7078

Home IV Program: 617-355-6499

Pulmonary Function Test: 617-355-7510

Page Operator: 617-355-6369

Hospital Main Number: 617-355-6000

CF Center web site:
www.childrenshospital.org/clinicalservices/Site1863/mainpageS1863P0.html

Email list serve:
Cfevents@childrens.harvard.edu

CF EVENTS

Children's Hospital Boston 2010 CFF Great Strides Team. Thank you to everyone who submitted t-shirt designs. Noor EL Shaar was the winner of the t-shirt design contest. Honorable mentions go to Stephanie Johnson, Samantha Johnson and The Hebert Family.

June 24th, 2010

13th Annual Jordan Classic Golf Tournament to benefit Children's CF Center families. Sponsored by the Outback Steakhouse at Brookmeadow Country Club Contact Mary Lyons 781-741-4156 (days); 781-738-0772 (evenings).

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