

## Parent Perceptions of the 23<sup>rd</sup> Annual North American Cystic Fibrosis Conference

*Deb Silvis*

Earlier this fall I was invited to attend the NACFC to participate as a parent panelist in a training session for health care professionals who are relatively new to serving individuals with cystic fibrosis and their families. I had always thought that this conference sounded very worthwhile, so it did not take me long to decide to pursue this opportunity. The conference was attended by thousands of people from many different countries and it exceeded my expectations. The NACFC is an impressive conference which was very well done.

The session that included the parent panel was called “Nuts and Bolts of CF Care.” The panel participants had never communicated with one another before, had limited experience with this kind of thing, and we were unsure of what to say. So collectively, we took a deep breath and “flew by the seat” of our pants! I hope that the panel was helpful!! While I learned many things listening to the various other presenters during the 8 hour Nuts and Bolts session, the best part for me was meeting the other parents on the panel. In the few minutes before and after the panel, we exchanged information about our local clinics, learning pill swallowing, parent support activities, how to continue treatments while traveling, the importance of exercise for “our” person with CF, and heard about the marriage and the first grandchild of a person’s adult daughter with CF, and many other things. There was so much to talk about that we had to continue our visit for a few hours after the session concluded! After we each introduced ourselves and shared a little bit of our “stories” the floor was open for workshop attendees to ask the panel members questions. One of the questions that the panelists were asked was “Should we keep on asking and saying the same things all of the time?,” to which the parent panelists shook their heads, sighed, and responded “Yes, keep on repeating things. On most days, the fact that you are repeating things does not insult us or make us angry. The repetition probably just needs to be done!” It was also nice that the parents had an opportunity to thank the professionals for choosing to help people with CF for their careers.

While the conference is primarily for health care providers and researchers focusing in the area of cystic fibrosis, parents and people with CF can benefit from attending this conference. There were numerous presentations that were of interest and understandable to me. I attended sessions focusing on the self care needs of teens and young adults, adherence in adolescents, transitioning to adult care clinics, exercise testing in the treatment and assessment of people with CF, and ways to address special nutritional needs of people with CF. In addition to presentations, I also enjoyed going through the exhibit hall and talking with various vendors there. I learned what “the posters” were and benefited from reading some of the research papers that presenters talked about. I brought home a huge bag of research information on topics of interest to me, books, videos, and samples of products that I am still making my way through. While eating lunch in the food court one day, I sat with the national coordinator of the TIGER study (which my daughter participated in), and people from Canada, the UK, and various states. We talked about differences in our countries health care systems. We compared costs of various “CF stuff”, and we compared how things are paid for. It was an interesting visit, which occurred completely by chance!

Because this NACFC was held on the 20th Anniversary of the discovery of the CF gene, I was among 3000+ people who heard Dr. Francis Collins, Director of the NIH, and Dr. Beall, Executive Director of the Cystic Fibrosis Foundation, speak. Dr. Collins was one of the 3 researchers who discovered the CF gene and he shared a fascinating and sometimes very comical presentation about that research “journey”.

Advancements in treatments and in the search for a cure for CF and many other medical conditions are occurring at a very rapid rate. I am confident that most people left the conference with feelings of optimism and hope that a cure for CF will be found.

## University of Wisconsin Cystic Fibrosis Center Specific Outcomes

Here is our center specific data for the calendar year 2008. There is publicly available data on all Cystic Fibrosis Centers available at the Cystic Fibrosis Foundation website ([www.cff.org](http://www.cff.org)). Data on that website is adjusted for attained age of patients, gender, pancreatic sufficiency, race/ethnicity, socio-economic status, and age of diagnosis. As of the writing of this newsletter, the CFF website has not yet been updated to include the 2008 data. We expect that data to be publicly available by late 2009 or early 2010. In the meantime, we now have our UW CF Center data for last year, and what follows in this article is the raw data reported to us for the calendar year 2008.

### Pediatric Center

A very useful measure of lung function is the FEV<sub>1</sub> percent predicted. For patients 6-12 years of age, the University of Wisconsin CF Center median FEV<sub>1</sub> percent predicted is 99.8%, which is 3.7% above the national average of 96.1%. For patients 13-17 years of age, the median FEV<sub>1</sub> percent predicted is 92% at UW compared to a national rate of 88.7%. For patients 6-17 years of age, the UW median FEV<sub>1</sub> percent predicted is 95.3% compared to a national average of 93.1%.

Our nutritional outcomes are expressed in terms of BMI (Body Mass Index) percentile. For pediatric patients 2-20 years of age, we strive for a BMI percentile of at least the 50<sup>th</sup> percentile. The median BMI percentile for CF patients 2-20 years of age at UW was 50.7 compared to a national average of 47.8.

### Adult Center

For patients 18-30 years of age, the median FEV<sub>1</sub> percent predicted at UW is 62.9% compared to a national average of 69.4%. For CF patients > 30 years of age, the median FEV<sub>1</sub> percent predicted at UW is 52.9% compared to a national average of 55.1%.

In adults over 20 years of age, one no longer uses BMI expressed as a percentile. In adult patients, BMI is expressed as the actual value with the units of kilograms per meter squared. The Cystic Fibrosis Foundation goal is that males should have a BMI of greater than or equal to 23 kilograms per meter squared and females should have a goal BMI greater than or equal to 22 kilograms per meter squared. In the UW Center, the percentage of adult patients who met those goals was 50% compared to the national average of 41.3%.

### Trends over the last four years

The following table shows data for the past four years. The UW CF Center specific data is shown first, followed by the national average in parenthesis. This allows you to see trends that are occurring both on a local and a national level.

|  | 2005 UW<br>(national average) | 2006 UW<br>(national average) | 2007 UW<br>(national average) | 2008 UW<br>(national average) |
|--|-------------------------------|-------------------------------|-------------------------------|-------------------------------|
| Median FEV <sub>1</sub> %<br>predicted 6-12 years                              | 92.6 (94)                     | 95.1 (95.1)                   | 95.2 (95.4)                   | 99.8 (96.1)                   |
| Median FEV <sub>1</sub> %<br>predicted 13-17 years                             | 87.3 (86)                     | 88.0 (87.4)                   | 91.4 (88.3)                   | 92 (88.7)                     |
| Median FEV <sub>1</sub> %<br>predicted 6-17 years                              | 90.5 (90.6)                   | 92.3 (92.1)                   | 94.5 (92.6)                   | 95.3 (93.1)                   |
| Median FEV <sub>1</sub> %<br>predicted 18-30 years                             | 57.5 (66.2)                   | 50.7 (68.1)                   | 58.1 (69.1)                   | 62.9 (69.4)                   |
| Median FEV <sub>1</sub> %<br>predicted ≥ 30 years                              | 62.3 (53)                     | 50.7 (54)                     | 50.9 (53.7)                   | 52.9 (55.1)                   |
| Median BMI<br>percentile 2-20 years  | 46.5 (44.8)                   | 51.6 (47.3)                   | 51.5 (47.5)                   | 50.7 (47.8)                   |
| Adults over 20 years<br>of age: % males with<br>BMI >23 and females<br>BMI >22 | 32.1 (37.6)                   | 36.7 (39.8)                   | 41.6 (41.1)                   | 50 (41.3)                     |

## CF Clinical Trials-Learn, Ask, Join

Do you or your child use Pulmozyme® or TOBI® or Azithromycin? Did you know that for those medications to be approved for use in CF, patients were required to participate in clinical trials? There were 968 patients who participated in the phase III Pulmozyme® clinical trial, there were 520 patients who participated in the TOBI® clinical trial and there were 185 patients who participated in the randomized controlled trial of azithromycin.

With so many new drugs in development, it is vital that patients participate in clinical trials. In fact, in the year 2006, approximately 3000 patients participated in clinical trials. Now with 20-30 new drugs in development, it has been estimated that this year and going forward, there will need to be 6000 patients participating in clinical trials.

### You can *learn* about CF clinical research at:

- <http://www.cff.org/research/ClinicalResearch/> Former NFL quarterback Boomer Esiason talks about clinical trials and his son Gunnar's participation.
- 1-877-8CF-JOIN This is the CF Foundation's toll free clinical trials hotline

### You can *ask* about CF clinical research at:

- Your or your child's clinic visits
- By calling the pediatric research coordinator, Linda Makhholm, at 608-262-0340, or the adult research coordinator, Sharen Wilson, at 608-263-0586

Please *join* a CF clinical trial.

## Seasonal And H1N1 (Swine) Influenza Update

In all previous years, we have made a recommendation that all persons with CF and other people in the home should receive the influenza vaccine. This year is no exception. However, this year, there are two influenza vaccines: the seasonal flu vaccine and the H1N1 vaccine. The seasonal flu vaccine provides protection against two strains of Influenza A and one strain of Influenza B.

As everyone knows, this year is different from previous years because of the H1N1 (previously called "swine flu") influenza outbreak. This virus was first detected in the spring 2009 and has now spread across the world in epidemic proportions. Vaccine manufacturers had already produced the seasonal influenza vaccine and it was therefore too late to include this novel H1N1 virus. Thus, there is a separate vaccine for the H1N1 virus.

People with CF should be immunized with both the seasonal and H1N1 vaccines. Although there are nasal forms of these vaccines, those should not be used in anyone with an underlying health condition such as CF. People with CF should receive the injectable vaccine.

The vaccine supply has been less than expected. From day-to-day, we do not know how much vaccine we will have available for patients. Please ask about the flu vaccines during your clinic visits, or you may call the pediatric clinic at 608-263-6420, or call the adult clinic at 608-263-7203.

In the meantime, you can protect against H1N1 flu is by minimizing the spread of germs. You can do this by:

- Washing your hands often with soap and water or using an alcohol-based hand gel.
- Covering your nose and mouth with a tissue when coughing or sneezing, then washing your hands.
- Avoiding touching your eyes, nose and mouth. Germs spread this way.
- Staying away from others if you are ill.



## Update From The North American Cystic Fibrosis Conference

Drug development continues to move forward (thanks to the participation of people with CF). Here is a brief overview of clinical trial results of drugs in the following categories:

### Anti-infective

Tobramycin inhalation powder (TIP) is a dry powder inhaled form of tobramycin. This is a similar delivery mechanism as some already-available dry powder inhaled medications for the treatment of asthma. The TIP is delivered in 4 to 6 minutes (compared to the 10-15 minute delivery time of TOBI®). In a phase III trial, the FEV<sub>1</sub> improved significantly in patients receiving TIP compared to those receiving placebo.

Arikace™ is a sustained release lipid formulation of amikacin for inhalation. This is delivered using the Pari eFlow® nebulizer with a delivery time of 10 to 13 minutes. The drug contained in liposomes (fat globules) is released slowly such that this drug is only given once per day. In phase II studies, the FEV<sub>1</sub> improved significantly in patients receiving the active drug compared to patients receiving placebo.

Aztreonam lysine for inhalation (Cayston®) is an anti-pseudomonas antibiotic that is delivered with the Pari Altera nebulizer (which uses eFlow® technology) with a delivery time of 2 to 3 minutes. This medication has received conditional approval in Europe and Canada and will be reviewed by the FDA anti-infective drugs advisory committee in December 2009. In phase III studies, this medication has resulted in improved pulmonary functions, a decrease in Pseudomonas colony counts and improved patient reported symptoms.

MP-376 is levofloxacin for inhalation. This is delivered using the Pari eFlow® nebulizer. Phase II studies have demonstrated an improvement in FEV<sub>1</sub> percent predicted.

Other anti-infective agents that are being studied include BAY Q3939 (an inhaled form of ciprofloxacin), GS 9310/11 (a combination antibiotic of fosfomycin and tobramycin) and KB001 (an antibody directed against Pseudomonas aeruginosa).

### CFTR modulation

VX-770 is an oral compound known as a CFTR potentiator. This may act upon the CFTR protein to allow the chloride channels to open. This medication has been shown to decrease sweat chloride values and improve pulmonary functions in patients with CF who have a G551D mutation. Studies are ongoing to determine the effect of this drug in patients with the G551D mutation and in patients with other mutations.

VX-809 is an oral compound known as a CFTR corrector. This may help to move defective CFTR protein to the proper place in the airway cell membrane and improve its function as a chloride channel.

Ataluren (formerly known as PTC124) is an orally available small molecule compound that may be useful in patients who have a CF mutation that ends in "X" (for example, the R553X mutation). These "nonsense" mutations lead to premature truncation of the CFTR protein. Ataluren appears to skip the stop signal (the "X"), and therefore the CFTR protein is manufactured.

If you are interested in more information and want to watch the plenary sessions of the North American Cystic Fibrosis Conference, please go to <http://www.cff.org/research/NACFC/>

## Pancreatic Enzyme Update

Mary Marcus, MS, RD, CSP

The federal Food and Drug Administration (FDA) now requires all drug companies who make pancreatic enzyme products to get their enzymes approved by the FDA by 2010.

Pancreatic enzymes were originally developed before the FDA required them to be tested in clinical trials to see how well they work. The FDA allowed the enzymes to be “grandfathered” products, so they could be available to patients. This new rule means that the FDA requires enzymes to meet the same standards of testing as any other new drug.

For enzymes to receive FDA approval, drug companies are holding clinical trials with people with pancreatic diseases, including CF, in order to confirm the safety and effectiveness of the enzymes. Several patients from the UW pediatric CF center recently participated in one of these clinical trials.

As of November 2009, 2 pancreatic enzyme products have received FDA approval. Creon, which is made by Solvay was approved this summer. Creon is now available as Creon 6000, Creon 12,000 and Creon 24,000. Zen-Pep, which is made by Eurand, is a new enzyme product on the market and received FDA approval this Fall. Zen-Pep is available as Zen-Pep 5000, Zen-Pep 10,000, Zen-Pep 15,000, and Zen-Pep 20,000.

People with CF who take any of the other brand name enzyme products, Pancrecarb (Digestive Care), Ultrase (Axcan), and Pancrease (McNeil), to digest their food still will be able to get these enzymes while the research occurs. Because pancreatic enzymes are so important for people with CF, these will still be available while the drug companies work on getting FDA approval. We will keep you updated as more enzymes are approved.

The UW CF Center and the CF Foundation support the FDA's decision because it will make enzymes better and help improve nutrition and digestion for people with CF. The CF Foundation worked with the FDA to ensure the best health for everyone with CF.

## KEEP IT UP – Tips to Prevent Weight Loss During the Holidays!

*Yes – the title is right!* We want to help you either gain weight or keep from losing weight during the holidays. Although you are probably used to hearing people talk about preventing weight gain during the holidays, many people with cystic fibrosis need to do the opposite. Most people with CF need to gain weight year round, including during the holidays. Please use this list to help you **keep the weight up** during the holiday season. Your nutrition status will be tip-top and your lungs will be healthy.

- Keep those enzymes handy! Always have extra in a purse, backpack, or pocket and at the homes of friends and family. If you are grazing (or eating throughout the day), remember to take extra enzymes every 45-60 minutes.
- Try some delicious high-calorie winter drinks – peppermint shakes, minty hot chocolate made with cream, alcohol-free egg nog, warm vanilla milk.
- Enjoy all the yummy samples at the grocery store. Remember to take those enzymes as you walk around the store, though.
- Eat some warm cookies straight from the oven.
- Avoid missing meals and snacks if you're out all day shopping. Pack snacks like trail mix, energy bars, nuts, dried fruit, and nut butter sandwiches.
- Add ice cream or whipped cream to pies and hot chocolate.
- Put extra butter on potatoes, rolls, veggies, and stuffing.
- Make room for a few more bites of your favorite food.
- Let your child/teen plan the holiday menu so he or she can have a much loved food to enjoy.
- Add chocolate chips, nuts, shredded coconut, dried milk powder, and/or dried fruit to breads, muffins, cookies, and cakes to boost calories.
- Serve fruit, veggies, and crackers with dips, nut butters, and cheese instead of plain.
- Make time for tube feedings or oral supplements. Skipping too many days can lead to weight loss.

### Peppermint Patty Shake (from Häagen-Dazs)

#### Ingredients

1 cup whole milk  
1 carton (14 ounces)  
Häagen-Dazs vanilla ice cream  
2 large chocolate covered peppermint patties



Combine all in a blender and mix until smooth.

Makes 3 servings (1 cup each)  
1 serving has 480 calories and 27 grams fat

Happy Holidays – Erin Seffrood, MS, RD, CSP

## **Become A Member Of The UW CF Center Parent Advisory Council**

As part of our commitment to provide the best care possible to patients with CF, the UW CF Center has formed a Parent Advisory Council. The purpose of the Parent Advisory Council is to work in partnership with UW CF Center faculty and staff to enhance the medical care and quality of life for children with CF. The Parent Advisory Council will meet regularly with Center staff to provide a parent perspective to various issues and projects of importance to patients, families and the CF Center. The time commitment involved will be a 90 minute meeting 4 times per year. We hope you will consider joining this important group. Please contact Kate Kowalski, MSSW, Family Involvement Coordinator, at [kmkowalski@pediatrics.wisc.edu](mailto:kmkowalski@pediatrics.wisc.edu) or 608.262.7457 with questions or to say that you will join us!

## **Family Mentors Needed For Graduate Student Training Program**

As part of their training experience, graduate students and pediatrics residents working with the Pediatric Pulmonary Center participate in a Family Mentor Experience. The purpose of the experience is to help trainees understand the day-to-day challenges involved with caring for a child with a chronic health condition. The Family Mentor Experience involves a trainee being matched with a mentor family, and spending two to three hours with the family in their home. The trainee talks with parents/family members about what it is like to care for a child with a chronic illness, and with the child about her/his experience of living with a chronic illness. Both the families and the trainees are provided with an orientation prior to the visit to assure that all understand the purpose and recommended guidelines. The parent orientation is typically done by phone. If you are interested in learning more about having your family become a mentor family, please contact Kate Kowalski, MSSW, Family Involvement Coordinator, at [kmkowalski@pediatrics.wisc.edu](mailto:kmkowalski@pediatrics.wisc.edu) or 608.262.7457.

## **Foundation Update**

As we reflect on the past year, we are so grateful for all of our volunteers who worked so hard to help to raise much needed funds for cystic fibrosis research. Even though we are all aware that the economy is down, we are thriving, thanks to you!

However, as we look forward to 2010, we know that it will face us with many new challenges! And in order to continue our successful journey of finding a control for CF, we need to have your help! If every family would just take a half hour to contact our local chapter and meet with us, we know that we can find a way to fit you into our plan to find a cure, which will fit your busy time schedules and abilities!! If you only have an hour a year, we can still use your help!!!

We have a lot of exciting things to share with you in 2010! The most exciting is that Dr. Bob Beall, President and CEO of the Cystic Fibrosis Foundation will be here in April to give our families a comprehensive overview of the recent medical advances and those we are working hard on. Who better to hear it from them "Mr. Cystic Fibrosis" himself!!! No one is more educated and knowledgeable than him!! And thanks to the CF Care Center for hosting Dr. Beall and the CF Foundation that night! The date will be either April 20 or 21... more details will be forthcoming!

Also, in 2010 we will introduce our new event, "Madison's Finest." This event will recognize up and coming professionals in the Madison area, while they raise money for our great cause. If you have someone to nominate for this event, we look forward to hearing from you! We are excited to move more into the corporate sector of Madison and the surrounding areas, and get the word out on our great cause.

But again, most importantly will be our GREAT STRIDES efforts! In Wisconsin, we raise over \$850,000 through our GREAT STRIDES events each year! Our dates are planned, and the only thing missing from our success is you! Please consider joining us this year if you have not in the past! It can truly be the difference in us finding our cure.

Thank you again for your dedication and determination to our great cause! We know that we are headed in the right direction, and just need your help to get there. Please contact the local chapter today to help us reach this next step! (In Madison- 608.298.9902 In Milwaukee- 262.798.2060 or by email [tearll@cff.org](mailto:tearll@cff.org))

Sincerely,  
*Tracy Earll*  
*Executive Director- CFF Wisconsin*

**New Pediatric Pulmonologist,  
Doctor D.B. Sanders**



Dr. Sanders was born and raised near Seattle, WA. He went to college at the University of Notre Dame and did his medical school in Chicago at Northwestern University. He stayed in Chicago for his pediatrics residency at Children's Memorial Hospital before returning to Seattle for his fellowship in pediatric pulmonology at Seattle Children's Hospital. He also has a Master's of Science in epidemiology from the University of Washington. He has conducted research on cystic fibrosis and will work with Phil Farrell's group on the ongoing CF research here at UW-Madison. His wife works at AFCH as a nurse.