

A Giant Leap Forward?

By Julie Desch, MD

Until very recently, therapy for CF has been directed at correcting the consequences of the defective CFTR protein (thinning mucus, improving clearance, treating infection, calming inflammation, improving nutrition, etc.). Yes, when the gene was discovered in 1989, there was a flurry of research in the area of gene therapy...finding a safe mechanism to insert a copy of the “normal” CFTR gene into the targeted cells and getting it to work. This proved to be quite an undertaking, and while there is still much being done in this field, the exciting research making news today is from companies like Vertex Pharmaceuticals. With the Vertex drugs and others like them, this is the first time that a therapy—a small-molecule, not gene therapy—is actually directed at trying to correct the defective protein.

In a recent article in “Xconomy”, Dr. Bonnie Ramsey (who should really be in the CF-caregiver Hall of

“When gene therapy was not proving to be wildly successful, some companies started to ask if the defective protein could be fixed.”

Fame) responded in part to a question about the Vertex drug VX-770, “Whether it turns out that Vertex is 100 percent successful or not, this is such a giant step forward, it’s like a man walking on the moon.” Walking on the moon.... I remember that day. It was huge. It is my mission with this article to try to explain as best I can what she is talking about.

To understand why VX-770 and its partner in crime VX-809 make such a giant leap forward for mankind, we first must have two small refresher courses.

CF Mutations 101

There are more than 1,600 known mutations of the gene that causes CF. We now know that each of these mutations fits into one of five

“classes.” Each member in a class of mutations causes a disturbance in the sequence from gene (DNA) to CFTR (protein) to functioning CFTR protein at the membrane of the cell (electrolyte transport into and out of cell) in characteristic ways.

In a Class 1 mutation, there is no synthesis of CFTR protein at all. Zilch. This can be the result of a “nonsense” mutation, where a STOP message is read on the mRNA (transcribed from the gene) somewhere along the line, and synthesis of the protein is aborted. Or, a Class 1 type of mutation can lead to a misread of the gene because of a “frameshift”. Think of a frameshift as what happens when you forget to answer ONE question on a multiple choice exam

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MILESTONES

Please share the milestones in your life with our readers. Your successes and achievements may serve as a source of motivation for others in need of an infusion of "positive mental attitude" in the pursuit of their goals. Send us a note specifying your "milestone." Include your name, age, address, and phone number. Mail to: **CF Roundtable, PO Box 1618, Gresham, OR 97030-0519. Or E-mail to: cfroundtable@usacfa.org**

ANNIVERSARIES

Birthday

Elizabeth Baroncelli
Middleboro, MA
51 on September 22, 2009

Arthur Herron
Carmichael, CA
28 on September 13, 2009

Lynn Williamson
Covington, LA
42 on September 18, 2009

Marjorie Winokur
New Hyde Park, NY
57 on August 29, 2009

Wedding

Elizabeth & Albert Baroncelli
Middleboro, MA
28 years on September 5, 2009

Kathi Novelli Clapham & Andrew Clapham
West Chester, PA
8 years on September 1, 2009

Andrea Eisenman & Steve Downey
New York, NY
1 year on September 13, 2009

David & Kathy Lantz
Howell, MI
1 year on September 27, 2009

Valerie Vandervort & Rick Boyer
Verdigris, OK
18 years on June 14, 2009

Brian and Virginia Weinstein
Plantation, FL
5 years on February 21, 2009

Lynn & Darren Williamson
Covington, LA
5 years on October 9, 2009

Transplant

Elizabeth Baroncelli, 50
Middleboro, MA
Bilateral lungs
2 years on March 26, 2009

David Lantz, 38
Howell, MI
Bilateral lungs
8 years on August 3, 2009

Valerie Vandervort, 37
Verdigris, OK
Bilateral lungs
8 years on October 4, 2009

LOOKING AHEAD

Please consider contributing to *CF Roundtable* by sharing some of the experiences of your life in writing. Read the **Focus** topics listed below and see if there are topics you might like writing about. In addition, humorous stories, articles on basic life experiences, short stories, art work, cartoons, and poetry would be greatly appreciated. We require that all submissions be original and unpublished. With your submission, please include a photo of yourself (as recent as possible) as well as your name, address and telephone number. Photos will be returned. Send all submissions to:

CF Roundtable, PO Box 1618, Gresham, OR 97030-0519. Or E-mail to: cfroundtable@usacfa.org

Autumn (current) 2009: Gender-related Problems in CF.

Winter (February) 2010: Diet and Nutrition. (Submissions due December 15, 2009) When you have CF, keeping a healthy body can be a trial. Do you have experience with maintaining an ideal weight and body mass? Do you have good ideas for ways to cook and/or eat a healthier diet? Please share your information with us.

Spring (May) 2010: Traveling For Work or Pleasure With CF. (Submissions due March 15, 2010) Traveling with CF can be a hassle. Have you learned any techniques to make travel easier? Do you have any suggestions for our readers? Please share your good ideas with us.

Summer (August) 2010: All Types of Support. (Submissions due June 15, 2010)



ASK THE ATTORNEY

Questions from Readers

By Beth Sufian, Esq.

The following questions are a compilation of questions asked by readers of CF Roundtable. Questions asked by readers are never disclosed without the agreement of the reader and information will never be published in a way that would allow anyone to identify the reader who asked the question. Nothing in this column is meant to be legal advice about your specific situation. If you have additional questions please contact the CF Legal Information Hotline at 1-800-622-0385. The Hotline provides free and confidential legal information to people with CF, their CF Center care teams, and their families. The Hotline is sponsored by the CF Foundation. The Hotline also can be reached by e-mail at CFlegal@cff.org.

1. If I get married will I lose my SSI and Medicaid benefits? I also have SSDI and Medicare. Will I lose those benefits?

Marriage does not mean automatic loss of SSI and Medicaid benefits. A person who receives SSI and Medicaid must stay within certain income and asset guidelines. If the person decides to marry, the future spouse's income from work and assets, (for example, money in the bank) must be within the guidelines set down by Social Security. A person should determine what the income cut-off is for a family of two in their state. Income guidelines vary according to state. The person with CF should determine if the future spouse has income and assets that will disqualify the spouse with CF for SSI and Medicaid benefits. If the future spouse's income and assets disqualify the person with CF from receiving SSI and Medicaid, the person with CF must make sure there will be a way to obtain insurance coverage under an employment-based policy through the future spouse's employer.

SSDI and Medicare are not affected by a spouse's income. However, if a person with CF is receiving both SSDI and SSI the person will have both Medicaid and Medicare. In such a situation, Medicaid should be paying for any



BETH SUFIAN

Medicare co-pays and Medicare premiums. If a person becomes ineligible for SSI and Medicaid, due to a new spouse's income and assets, typically the person will not be eligible for most Medicare subsidies that help pay for Medicare premiums and co-pays.

If a new spouse has employer-based insurance, insurance coverage offered by his or her employer, and the spouse with CF enrolls in it as a dependent, then the private coverage will typically be primary and Medicare secondary. It would depend on how many employees are employed by the employer. Before

marriage, the person with CF should make sure there is a good understanding of costs that the person with CF will be responsible for under the private insurance policy. Sometimes people who have had Medicaid and have not had to pay anything out-of-pocket for medication and treatment are shocked to find out how much out-of-pocket costs a person who has private insurance coverage will have to pay for medication and treatment. Understand how much the co-pays and other costs are under the private insurance plan.

2. I am on my parents' health insurance policy. It is a very good policy. But when I turn 22 next month I will no longer be eligible for coverage. What can I do to try to keep the coverage?

Most insurance policies have something called a "limiting age" for dependent children covered under the policy. When a dependent child reaches a certain age he can no longer be covered by his parent under the parent's health insurance policy. Each policy sets out its own limiting age. Some policies set the limiting age at 18, others at 22. A person should read the policy and know what the limiting age for dependent coverage is under the policy's terms. The adult child may be able to stay on the parent's policy after reaching the limiting age if the adult child is a full-time student. Again, each policy has different age limits related to full-time student status. Some policies stop coverage at a specific age regardless of whether the child is a full-time student.

Under COBRA, a child who reaches a limiting age on his/her parent's policy is allowed to extend cov-

erage for 36 months. However, the parent must pay the full premium. If the employer has been paying part, or all, of the insurance premium, then the parent will have to pay the full premium. COBRA is a federal law and mandates an extension of coverage in five situations. COBRA applies to employers who have 20 or more employees.

If the adult child reaches a limiting age on the policy and is incapable of self-support, most states require the insurance coverage under a parent's policy to continue. If you would like to know if your state has such a law, contact the CF Legal Information Hotline at 1-800-622-0385. These state laws are extremely helpful for the person with CF who

has excellent insurance coverage under a parent's policy and is not working full-time with access to insurance coverage from his/her own employer. The "disabled adult child" laws do NOT require that the adult child be deemed disabled under Social Security rules. The laws require only that the treating physician complete a form that states the adult child is incapable of self-support due to CF. Many people with CF perform numerous time-consuming medical treatments each day making it hard to go to college full-time. The disabled adult child law allows the adult with CF to continue on a parent's policy and work part-time or go to school part-time or not work at all. The adult child can stay on the par-

ent's policy until he is capable of self-support. If he is never capable of self-support, due to his health, he stays on the policy. If the parent's employer has been paying the full premium the employer continues to do so.

Once an adult child goes off the parent's policy, most policies will not let him back on the policy if he becomes incapable of self-support at a later time. So an adult with CF should be sure he can sustain full time employment before he leaves his parent's insurance policy. ▲

Beth is 44 and has CF. She is a Director of USACFA. Her contact information is on page 2. You may send CF-related questions of a legal nature to: bsufian@usacfa.org.

In These Trying Times

We know that some people may be going through a hard time with their employment and/or their medical insurance. We are letting you know about a program Genentech is offering called Pulmozyme Access Solutions. Remember, even when times are hard, it's important to keep taking your medication as prescribed by your physician. Focusing on your health is the best way to be there for your family.

Pulmozyme Access Solutions is Genentech's commitment to cystic fibrosis patients. They are here to help find a way for you to get the Pulmozyme your doctor has prescribed.

■ **Do you have questions about your insurance coverage for Pulmozyme?** They can help you navigate benefits, coverage or reimbursement issues.

■ **Have you recently started Pulmozyme or changed insurance companies?** The StarteRx Kit is a free, 30-day supply of Pulmozyme, nebulizer and educational materials provided to patients initiating therapy while insurance coverage is ascertained.

■ **Do you need help with your co-pay for Pulmozyme?** They can refer you to independent, non-profit organizations that provide co-pay assistance and help you with the application process.*

■ **Are you uninsured, has your insurance company denied coverage for Pulmozyme or have you met your annual or lifetime insurance cap?** Genentech Access To Care Foundation provides Pulmozyme free of charge for eligible patients without insurance coverage.

If you answered "yes" to any of these questions, Genentech specialists can help you or someone you know. Call (800) 690-3023 from 6 a.m. to 5 p.m. PT, Monday-Friday, or visit PulmozymeAccessSolutions.com anytime. Check them out if you are in need. They are here to help us out.

*Genentech cannot guarantee co-pay assistance once you have been referred by Pulmozyme Access Solutions. The independent, nonprofit organizations to which patients are referred each have their own criteria regarding eligibility, including financial eligibility. Genentech does not influence or control the operations of these independent, nonprofit organizations, but Pulmozyme Access Solutions can help you navigate the process of seeking co-pay assistance by referring you to an appropriate organization and by assisting with the application process.



SPEEDING PAST 50...

Autumn Odds and Ends

By Kathy Russell

I can't believe that another summer is gone. Time really does go faster as you age. It seems as if it was only yesterday that we were enjoying the first crocuses of the year and now the autumn crocuses are blooming. I hope that this winter will be easier than last winter was. We don't need any more trees falling and I don't want to get any more respiratory infections. (After fighting bugs all winter and spring, I spent six weeks of the summer on antibiotics; and that is unusual for me.)

We took a vacation to the beautiful Oregon coast in July. We had six nights of breathing very clean air that had a nice salt tinge to it. My lungs said a huge "Thank you" for that. I always breathe better at the coast. I think it is a combination of the salt air and the fact that the air has come thousands of miles across the Pacific, getting "scrubbed" all the way, and isn't full of auto exhaust and industrial pollutants. I am able to walk easier and farther, and I definitely sleep better with that cleaner air.

It was very nice to have a vacation where we had no pressing engagements. We weren't planning on visiting anyone and we were not doing anything that had anything to do with CF. Often, we take trips for CF Conferences or other CF-related doings and it was nice to not have to think of CF all the time.

That brings up an issue that I have heard bemoaned repeatedly...not being able to get a "day off from CF". Usually, it is a caregiver who is bemoaning the fact. Sometimes, I remind them that their child (or grand-

child or spouse or whoever it is that has CF) never gets a day off from it. CF is as much a part of our lives as breathing or eating. (Oh! Good one, Kathy!) We never can *really* get away from CF. Even if we choose to take some time off from some of our treatments or meds; we still have CF...all the time.

If you are like I am, you are afraid

to miss a treatment or a med because, if you were to get sick, you would feel so guilty about having been less than fully compliant. Even though we don't get a cookie or a lollipop for doing our meds and treatments, we still feel we should be rewarded with good (or at least better) health for doing them. In fact, there is no guaranteed reward.

On the other hand, if we don't do our treatments and we skip our meds, we can be fairly certain that we will get sick or feel less well. Such a deal!

Many of us try to multitask while doing treatments. It might be interesting to know how many people who have CF are sucking on a nebulizer or wearing a vest, while reading e-mails or watching

TV. I imagine that number would be significant. I think this helps us to feel as if we aren't "wasting" our time with our treatments. (I know, it isn't wasting time, but I also know that it can sure feel like it – sometimes – when half of one's day is taken up with CF.)

The amount of time that taking care of CF entails isn't apt to change for the better, in the near future. There are so few (relatively speaking) people who have CF that there isn't a big incentive to get anyone to find ways to free up more of our time. If I am incorrect in thinking that, then perhaps someone will write to us and tell us what they are doing to address that issue. Oh, well, such is life.

Being an older person and being on Medicare, I am concerned about the costs of healthcare. I am very fortunate because I have excellent medical insurance that supplements

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KATHY RUSSELL

Medicare. That insurance is not guaranteed, however. At any time, the company (my husband's former employer) could decide to stop making it available to us. The premiums keep going up – at a rate faster than a speeding jet! – and we keep paying a larger percentage of our income to maintain it. In the past few years, our premiums have gone up more than 500%. The deductible (the amount we have to pay, out-of-pocket, before the insurance pays anything) has increased 600%. Our co-pays for prescriptions have risen 2400%! We try to be sensitive to costs and to not overuse our benefits, but there are times when we are unable to avoid expensive health costs — think Pulmozyme or antibiotics, for instance. It causes enough anxiety that I find that I have to stop thinking about it and just let life happen. So far, so good.

Wouldn't it be lovely to have an affordable healthcare system that would cover us so that we wouldn't have to feel such concern? What an ideal system that would be. Imagine if we could go to our CF doctors and get all the tests and treatments that the doc would like us to have and not have to worry about some insurance company saying, "Oh, we won't cover that." Wouldn't that be nice? How about being able to get the medicines my doctor orders, without having to go through an "appeals process" to get exactly what was ordered? I can dream.

I find that I feel resentment toward the companies that have people who question everything that is ordered for me; especially since those people are not necessarily medically trained. Often they have no knowledge of anything except how to say, "No!". I understand that they work from a list of "approved" meds and treatments, but I know that that list was made with no thought of someone having CF. I get tired of having to explain everything to each person, every time. I also get tired

of having to remind them that I am paying (and paying plenty) for their services, when they act as if my coverage is a gift. Ah, the joys of dealing with big corporations. Maybe someday our system will improve.

With the coming of autumn, I revert back to my "country" upbringing where we were so busy "putting foods by" and getting fuel stored for the coming winter. Paul, my husband, makes sure that we have a more than adequate supply of firewood for the woodstove and oil for the furnace. He cleans the chimneys and gutters, and checks the antifreeze in the vehicles. I preserve foods for the pantry.

I find that I am not canning as much as I used to. It takes a lot of energy to do all the preparation for canning and a lot of standing and working to get it all done properly. I no longer have that much energy. So, I can less, but I still can the things that we really like. Seeing the jars of tomatoes, soups and fruits on the pantry shelves gives me a real sense of accomplishment and security.

There is another real plus to doing one's own food preservation and that is: knowing exactly what is in each container. I don't have to worry about dyes, additives or preservatives in my food. When I can tuna, I don't add anything to it, not even water or salt. It is just clean albacore filets and it is delicious. No commercially prepared tuna can compete. My tomatoes may have some water added, but that is all. They are delicious to eat, right out of the jars. The fruits have a very light sugar syrup added that enhances their flavors without covering them up. All of them are worth the effort.

Autumn also brings shorter days. I find that using my "daylight" lamps really helps me to avoid experiencing seasonal affective disorder. The bright light helps to fool my body into not realizing how short the days are getting. Anything that I can do to help

avoid depression is wonderful. Depression is so common in people who have chronic illnesses, and those with CF are not immune to it. I prefer to treat things with non-invasive methods, rather than taking pills, if at all possible. The lights seem to make a big enough difference for me that I don't need pills to ward off depression. That doesn't mean that I never have days when I feel depressed; it just means that they don't escalate into something worse.

Another phenomenon of autumn is flu season. I will get two flu shots, as I usually do. The first injection is in September or October and the second will be three months later, to last through the second wave of flu season. In addition, I will get at least one H1N1 flu shot. My husband and I discussed this, at some length, with our doctor. He recommends that we each get two of each. We will see if that is how it turns out. We need to remember that flu is a killer and that it can be very hard on us. Please be sure to discuss this with your doctor. It could save your life.

As I look out at the trees, I see that the holly berries still are green as are all the leaves on the trees. But I notice that the "airplane" seed cases on the big-leaf maples are brown. The black walnuts are falling and all the English walnuts and hazel nuts have been stolen by the squirrels for their winter food supply. And so life goes. The cycle continues. Much as I hate to see summer leave, I look forward to the wonderful colors of autumn and the cups of hot chocolate or tea that help to keep me warm through the coming months. If only it didn't keep going faster and faster.

Until next time, stay healthy and happy. ▲

Kathy is 65 and has CF. She is a Director of USACFA. Her contact information is on page 2.



A DEEP BREATH IN We All Have a Story

Debbie Ajini

CF is teaching me to better understand that every single person has their own struggles and their own story. Sometimes when you have a disease like CF, it is very easy to get tunnel vision. By that I mean that sometimes when we are not feeling good and dealing with doctors, meds, etc., we can forget there is a whole world of people out there. They all have joy and they all have pain. They all have a story and it is true.

When I get really sick, I typically narrow my focus to just taking care of myself and getting better. That is fine. But also in the midst of being sick I will sometimes have a good ol' pity party. I wonder why people don't call. I wonder if they know how difficult things are and how bad I feel. I ask, or rather expect, people to understand how difficult it is to have CF. I wonder why they don't "get" how difficult it is to breathe. I think these thoughts are perfectly normal and acceptable. But there comes a point for me, as I am starting to get better, when I realize life has gone on around me. And sometimes that means people I love have faced their own hardships. And most certainly people I don't know have faced hardships. People have had joyous things happen to them. And it reminds me there is more to this world than me and my struggles!

The fact that I am facing a transplant could be just as scary for me as it is for a friend who may be facing unemployment. If they have had different life experiences and dealt with different events in their life than I have, then to them, at that time, losing their job may be the worst thing

ever. And because that is how *they* feel, that makes it TRUE. If we wrote down all the bad things that can possibly happen to anyone there will always be a long line of progressively worse things that a person can face. But the only ones that really matter are the ones we face ourselves and then the ones the people we love have to face.

I know life only from my experiences and perspective. The same is true for you and everyone we know, and for that matter, everyone we don't know. What is very real to me right now are the big losses I have faced this past year. My mom and a very dear aunt both died within the past year. And in the first week of September, my husband and I had to

put our beloved dog, Max, to sleep after 12 and a half years of life with us. That is my reality right now. But the reality for some dear friends of mine is dealing with fertility issues. We are both experiencing things that are very real. The key is understanding, with compassion, that no one issue is better or worse than the other. I do not believe in dismissing someone else's current life struggles because mine may appear harder or worse.

As I mentioned earlier, I tend to get tunnel vision when I am sick. I have become more aware of this, so when I do get sick I make an extra effort to connect to someone else's world and to remember that everyone has struggles and pain. To be clear, this is not to compare our woes as to

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who has it worse but to support each other through the tough times. I firmly believe that the struggles one goes through are the toughest and most real only for them. We could never measure their pain, luck,

indifference or other emotions involved. Going into someone else's circle reminds me that we all are more alike than we think. What brings me the most joy is being able to step outside of my bubble and walk over to a friend dealing with her own stuff and offer to listen, give her a hug or send a card.

We all have our own universe. That is okay. I just try to remember my own universe can't revolve without connecting to another person's universe. Imagine the cogs of a clock. No one piece is more important than the others because they all need each other to move. But each is the most important because without it the



DEBBIE AJINI

clock would stop. So it is with people. The things we deal with individually are just as important as how we relate to those around us and what they are dealing with.

Having CF has given me compassion for how it feels to be alone – to not have anyone understand you. I try to use that knowledge and help others.

I know it can be easier to sit in our own circle and wait for people to come in. But I also know they may not know how to come in or what to say. Sometimes we have to invite them in and tell them what we need. In turn that can teach us to NOT wait to be invited into someone else's circle. We know what it is like; we know it can be

hard to ask for help. So offer it. Be the teacher. See each person for their unique story just as you want someone to see you for yours. Because we all have one and they all are true. ▲

Debbie is 39 and has CF. She is a Director of USACFA. Her contact information is on page 2.

Information from the Internet...

Compiled By Laura Tillman

This issue brings a potpourri of articles from the Internet

FYI/NEWS RELEASES

Cystic fibrosis treatments may have unseen long-term benefits

Cystic fibrosis medicines that help to break down mucus in the lungs may carry an unexpected long-term benefit. The treatments not only help breathing in the short term - they may also make lung infections develop to be less harmful in the long run. Scientists studied how bacteria which infect the lungs of cystic fibrosis patients gather nutrients from their surroundings. The work builds on the knowledge that most bacteria cooperate to scavenge what they need from their environment, but some bacteria do not actively hunt, instead stealing nutrients from neighboring bacteria. Scientists found that in a viscous environment, similar to thick mucus, the cooperating type of bacteria is most common. However, in a more liquid environment - similar to mucus having been broken down by medicine - the number of thieving bacteria increases, eventually outnumbering the scavenging type. In this environment, because the thieving bacteria are less adept at obtaining food,

the bacterial growth slows down. The results suggest that liquefying lung mucus would be expected to limit the impact of infection in cystic fibrosis.

http://insciences.org/article.php?article_id=6138

Cystic Fibrosis Treatment May Cause Hearing Loss

A common antibacterial treatment for cystic fibrosis can cause sensorineural hearing loss. Researchers reviewed the medical records of 50 cystic fibrosis patients treated over a 13-year period at Children's Hospital Boston and found that seven (14 percent) of them suffered from sensorineural hearing loss. Of those seven patients, 43 percent had received more than 10 courses of aminoglycosides intravenously. Patients who underwent more than five treatments with nasal irrigation of aminoglycosides were also at risk for sensorineural hearing loss. Pulmonary and sinonasal infections are common in cystic fibrosis patients. Because of their potency against bacteria, aminoglycosides are often given to cystic fibrosis patients, even though the treatments

are known to cause side effects such as hair cell loss, which leads to hearing loss. Cystic fibrosis patients should have regular hearing tests that specifically assess sensorineural hearing loss, especially when patients have undergone repeated courses of systemic or intranasal aminoglycoside treatments.

The study appears in the July issue of the journal *Otolaryngology—Head and Neck Surgery*.

<http://healthfinder.gov/news/print-newsstory.aspx?docID=628535>

Common Cold Virus Efficiently Delivers Corrected Gene To Cystic Fibrosis Cells

University of North Carolina at Chapel Hill School of Medicine scientists have found what may be the most efficient way to deliver a corrected gene to lung cells collected from cystic fibrosis patients. They also showed that it may take this high level of efficiency for cystic fibrosis (CF) patients to see any benefit from gene therapy. Using parainfluenza virus, one of the viruses that causes common colds, the UNC scientists found that delivery of a corrected version of the CFTR gene to 25 percent of cells grown in a tissue culture model that resembles the lining of the human airways was sufficient to restore normal function back to the tissue. Now the researchers must work to ensure the safety of the delivery system. In a pleasant surprise, simply adding the CFTR gene to the

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From the Experts...

This is the first of what we hope will be a series of articles written by various experts on a variety of subjects that are of interest to people who have CF, or who deal with it.

Cystic Fibrosis And Lung Transplantation

By Ramsey Hachem, MD
Transplant Pulmonologist
Washington University School
of Medicine
St. Louis, MO

Cystic fibrosis (CF) is a multi-system genetic disorder that afflicts approximately 1 in every 2000 to 3000 live births in the United States. The gastrointestinal tract, the liver, sweat glands, and the reproductive tract are usually involved, but progressive lung disease is the primary cause of morbidity and the leading cause of death.

Bronchiectasis is characterized by the accumulation of thick mucous in the conducting airways resulting in airway obstruction and persistent infection. The clinical manifestations include a productive cough and chest congestion, and the natural history of the disease is accentuated by recurrent exacerbations and a progressive decline in pulmonary function.

Over the past 20 years, treatments focusing on airway clearance, suppression of airway inflammation, and nutritional support have delayed disease progression and slowed the rate of decline in lung function. Indeed, the expected survival has progressively improved, and the median survival is currently 37 years. Lung transplantation is the ultimate, albeit imperfect, treatment strategy for CF-related bronchiectasis.



RAMSEY HACHEM, MD

In general, lung transplantation is reserved as a last treatment option for patients with end-stage lung disease because outcomes after transplantation are disappointing, and the median survival is approximately five years. Thus, determining the right time for transplantation is critical to maximize the transplant benefit. Based on data acquired in the 1970s and 1980s, the median survival for patients with CF is approximately two years when the FEV₁ (forced expiratory volume in 1 second) reaches 30% of predicted, and this has been the main threshold to refer a patient for transplant evaluation. However, other important variables are considered, including clinical course, frequency and severity of exacerbations, body weight, PaCO₂ (partial pressure of arterial CO₂), pulmonary hypertension, and the flow of supplemental oxygen required. Nonetheless, there is usually a tight correlation between these clinical

factors, and the FEV₁ remains the best single predictor of outcome.

In the past, lungs were allocated based on waiting time, and most patients with CF were listed for transplantation when their FEV₁ was approximately 30% of predicted in anticipation of an extended waiting time. However, in 2005 the lung allocation system in the United States changed. Under the new policy, priority for transplantation is determined by medical urgency and expected outcome after transplantation.

A lung allocation score (LAS) is calculated for each patient based on the diagnosis and other clinical variables. This score is meant to reflect the expected survival on the waiting list and the predicted survival after transplantation, so that the transplant benefit is maximized. Under this system, there is no advantage to early listing since waiting time is irrelevant and priority for transplantation is determined by the LAS. However, early referral to a transplant program is still justified to prepare medically and emotionally for the transplant, familiarize the patient and their family with the transplant physicians and coordinators, and provide transplant education.

Most patients with CF are suitable candidates for transplantation since they are typically young and have few serious comorbidities. However, infection with *Burkholderia*, especially genomovar III, is considered an absolute contraindication to transplantation at many centers. Patients infected with genomovar III before

transplantation have had significantly worse survival after transplantation because of serious infections with the organism. The impact of infection with other genomovars on post-transplant outcomes is unclear and some transplant programs accept patients infected with these organisms. Otherwise, intubation and invasive mechanical ventilation is the other major complication for patients with CF that impacts candidacy for transplantation. Most centers today do not consider invasive ventilation an absolute contraindication, but there is usually a time window of opportunity as other complications develop and increase the risk of a poor outcome after transplantation.

At all time points after transplantation, infection and rejection are the most frequent serious complications. In the early post-operative period, infection, especially pneumonia, is common and a leading cause of early death. Chronic rejection becomes prevalent beyond the first year after transplantation and is the other leading cause of late mortality. Nonetheless, patients with CF have a comparatively good outcome after transplantation. In fact, the 5-year survival is 55% and the 10-year survival is 38%. While these results are still disappointing, they are significantly better than for most other lung diseases including pulmonary fibrosis, pulmonary hypertension, and chronic obstructive pulmonary disease (COPD). ▲



CLUB CF ONLINE

The focus of Club CF is: LIVING BREATHING SUCCEEDING. Club CF wants those who have CF or are affected by the disease to see that, despite all the challenges that come along with cystic fibrosis, it is possible to live a happy and successful life.

Club CF shows how people in different age groups (20+, 30+, 40+, 50+, 60+, caregivers) are succeeding. Through Club CF, people can give hope and inspiration to those who are hesitant or nervous about what lies ahead of them.

People with CF are succeeding and making a difference in the world in high school, college, sports, careers, relationships, starting a family, post transplant, and disability. If you are one of the many people who are LIVING BREATHING SUCCEEDING, join Club CF and show the world what you have done! To learn more, please visit us online at: www.clubcysticfibrosis.com

Club CF is sponsored by The Boomer Esiason Foundation, which is committed to showing the world that people with CF are living longer & fuller lives, and by generous support from Genentech.



Announcements



VOLUNTEERS NEEDED FOR STUDIES AT NIH

The Pulmonary-Critical Care Medicine Branch of the Department of Health & Human Services, National Institutes of Health (NIH), National Heart, Lung, and Blood Institute, in Bethesda, Maryland is conducting a **research study to evaluate the role of bacterial products involved in lung disease in cystic fibrosis**. We are looking for individuals with cystic fibrosis and *Pseudomonas aeruginosa*. The participants will be seen at the NIH. They will have blood drawn (around 2 tablespoons) and also have a sputum sample collected. The participants with CF will be paid \$50.00 for taking part in this study. We will pay for the transportation of patients who do not live in the local area. If you have CF, are at least 18 years old, have *Pseudomonas aeruginosa* and are interested in more information about this study, please call us collect at (301) 496-3632 or send E-mail to: barnesp@nih.gov.

A **research study of hereditary factors associated with cystic fibrosis and other lung diseases** is being conducted at the Department of Health & Human Services, National Institutes of Health (NIH), National Heart, Lung, and Blood Institute in Bethesda, Maryland. Participants will be admitted for an overnight stay at the NIH to have blood drawn, a PFT, chest x-rays, and EKG. Assistance with travel costs as well as a \$150 stipend will be provided. If you have CF, are 18 years of age or older, and are interested in participating in this study, please call us collect at (301) 496-3632, or send E-mail to: barnesp@nih.gov.

We are looking for individuals with cystic fibrosis who previously participated in NIH studies. If you have taken part in an NIH study, please call the toll free number: 1-877-644-5864 and select #3 on the menu; or send an E-mail to: barnesp@nih.gov.



FOCUS TOPIC

GENDER-RELATED PROBLEMS IN CF

Chronicles of a CF Boy

By Eric Hyman

As a male with CF, I figure I could contribute something to this topic. It'll be interesting to see if all the other men repeat the same sentiments. So growing up with CF was cool at times. As an elementary school kid, I sometimes enjoyed the special treatment and attention resulting from being a cute kid with a chronic illness. As I got into middle-school all the boys

who can beat up whom. As a frail tyke I wasn't beating up anybody, but to get some respect I had to get some big friends who could beat up everyone else, which I did. Good thing I was somewhat funny and into "gangsta rap" or I would've been on my own in 1990. My mom took me to an endocrinologist at some point that year and the question was whether to treat me with some growth hormone. It was a big debate, but I think the

ity, I had assumed I was in the 97 percent of the CF male group but just had to check. One doctor reported the results to me, while I was an inpatient, with a very solemn look and an apology confirming that I'm in the sterile majority. I had already accepted the odds and told her not to worry; at least I don't have to worry about birth control. This wasn't a blow to me or my male ego; however, it seems one of the social criteria for being masculine is being able to impregnate with ease. So I'm not embarrassed by it, but it's not something I announce to many people when the subject of children comes up because I get the sense that I should be ashamed. I imagine this would be much more dramatic news if I hadn't known the odds since I was 13. At this point I don't think parenting is for me. I'm happy just being an uncle; however, I recognize my priorities may change in the future and that there are some options available to become a father.

Going back to appearance, I know this doesn't apply to all, but I think I'm definitely a model of how CF affects a man. I'm grateful for the most part that I can hide my disease and move through the crowds of society without getting gawked at, like someone with a physical deformity. However I'm 32 years old now, still have a very frail frame, still getting carded and don't feel very manly at all.

At previous jobs and various settings in life I experienced lots of criticism and jokes at my expense. It's typically not socially acceptable to make fun of overweight people to their face, but skinny guys are fair game. When I was a college student, a hospital employee guessed my age around 12-14 and was amazed that I



ERIC HYMAN KAYAKING.

around me started suddenly sprouting and looking like men. Eighth grade came around and I was still about 4 feet 7 inches. I was dating a girl who was 5 feet 5 inches whom I took to my pseudo-bar mitzvah. (I got the party without learning Hebrew. Sweet deal; thanks to grandma. Probably also partially related to the CF I drew sympathy)

Anyway, every few years, I look back on the video or picture of that party with the tall girl – boy, does that look awkward. Of course, it made for easy jokes for those around me. The social hierarchy of 8th grade boys became who's cooler, tougher and

end-result was we figured I'd grow eventually. I'm 5 feet 7 inches now.

Around that time I also was educating myself about CF. There were booklets published on several CF subjects. (I believe it was called the Baylor Project. My mom worked at the CF clinic so she was well connected to any materials available.) The booklets taught me a good bit about the questions I never thought to ask. They confirmed it was normal to hit puberty a few years late, and told me about CF reproductive issues, which I had never heard before.

By the time I got tested for steril-

was 20. Granted, he was probably very bad at that game.

When I worked at a power plant, between semesters, I always felt like this little kid with a hardhat walking around the big burly men. I became friendly with an EPA representative who was helping us out, and learned he had a friend with CF who wouldn't tell anyone about the CF. He said all the friends assumed he had AIDS, when he was getting sicker. I started realizing that may be an assumption that people make about me. I learned that a couple of people thought I was gay – I'm guessing because of my non-masculine appearance.

I think I'm finally starting to look at least 25 since that's what most guess me as. My sister and others comment, "Well, when you get even older, you'll appreciate it because you'll still look young." I point out that it doesn't work like that with a man. I don't want to look like a kid representing myself as a professional. It's been better lately, but as a professional engineer I occasionally attend meetings with architects, contractors, other consultants and represent my company as an expert. Typically everyone else is much bigger and older than I am, so I get a little self-conscious that I look like a kid telling these professionals how they should design and build their buildings. Occasionally, people are a bit condescending, but mostly they hide it.

Of course, one other big aspect to being a man with CF (how could I forget) is being vulnerable and allowing others to take care of me. At the CF retreat, we actually had a male rap session where this was one of the topics that came up. Maybe this is another reason why I never really felt masculine. I think I've accepted that I'm vulnerable and sometimes need to ask for help. Though I don't do it often, I know I don't have control of every situation.

This past April my girlfriend was on spring break and we had all the

reservations set up to go to Disneyland. We were packed and ready to go. After finishing dinner at a restaurant nearby I had a big hemoptysis episode. We decided to go back to my apartment and I took some extra vitamins, probiotics and cayenne, which sometimes help prevent more bleeding. We got home and it turned into a massive episode. My girlfriend sat by me on the tub stroking my back. As I spit blood into the toilet, she took care of me, canceled the hotel and was supportive of canceling the plans. Later she postponed a flight so she could drive me

coworkers. A friend of mine pointed out that I don't have to strive for promotions and become a principal and all those other goals that would please me and my boss. He also pointed out that not everyone should be a boss. Some people can just be workers and can still do a job well while being just a lower level engineer.

So I did have the talk with my boss and I am using my CF for special considerations. I know there were some rumors behind my back about me taking advantage and using my disease. One person finally asked me if CF does take extra time out of my

“I hate being the cause of drama and not being in control. It's not the male role I feel is the norm.”

to the hospital and hang out with me. I hate being the cause of drama and not being in control. It's not the male role I feel is the norm.

A few years ago, I was debating whether to start working at part-time status. Before that, I was a very dedicated hard worker. I'd work sometimes 14 days straight, staying past 9 pm most nights, sometimes past 11 pm, because I wanted to do my job well. I could feel the stress taking a toll on my lungs and well-being. When I thought I was alone, I would have some really bad coughing fits – deep and rattle-y coughs – then I'd hear a supervisor's voice yelling out, "Are you okay?" and telling me that I should go home.

I was a career man. I wanted to excel at my job. The stress eventually became overbearing and I discovered a way to work part-time, without too much wage loss. This would be a blow to my career-man ego; going to my boss and pleading for part-time status because I'm too vulnerable, sick and lack the energy to keep up with my

day. I summarized mostly the neb treatments, eating well and trying to get in some exercise.

All in all, CF has left me feeling like a boy not quite fit for parts of this testosterone-enriched world. I've learned to accept and adapt to my emasculating features. I moved out to San Francisco where people are a bit more accepting of a short and skinny, vegetarian, tree-hugging pacifist - as opposed to my hometown of Philly, where cheese-steaks and sports are king. I'm a bit intimidated, sometimes, going out into the world of man, but I've managed to find my niche in this world. I'm happy to be growing up within a generation where the traditional roles and expectations of men and women have become a bit fuzzy and, of course, in a time of medical breakthroughs in CF, where I can be old enough to be a man and complain about this stuff. ▲

Eric is 32 and has CF. He lives in San Diego, California. You may contact him at: erocks77@gmail.com.



Female Trouble

By Andrea Eisenman

There are many things I have learned about having CF and being a woman. One main issue is hormones and their fluctuation during a woman's cycle. Because women's level of estrogen fluctuates during a period of around 28 days, it can cause many women to bloat, have migraines, diarrhea, constipation, congestion, increase in inflammation, low blood sugars, anemia, hemoptysis, mood swings—the works. When a woman has CF, these seemingly innocuous conditions can worsen the daily symptoms of CF. I learned many of these things from other women who have CF and some by just taking notice of my own body.

In my late 20s I had started to notice that during certain times of the month my lungs and sinuses got more congested. Soon after, I finally met some other women with CF who experienced the same thing. As estrogen starts to decrease and progesterone increases several days before menstruation bleeding starts, it can exacerbate the symptoms of lung disease, such as CF and asthma. My friends and I surmise that this drop in estrogen is likely responsible for our myriad of symptoms. The rise in progesterone during this time may also play a significant part.

In my case, during this drop in estrogen and increase in progesterone, my sinus/lung tissue would become inflamed and my lungs more congested; then I would usually get a cold or an exacerbation. This meant I would need heavy-duty oral antibiotics or home IVs. It also caused more lung bleeds for me at this particular time. This nose-dive of estrogen can be responsible for yeast infections, (sinus) migraines, worsening of arthri-

tis and may play a part in osteopenia as well.

After learning this new information, I wanted to minimize my getting a cold or hemoptysis every month; so, when I knew I was starting to feel more mucus in my lungs, and I was pre-menstrual, I would do an extra inhalation of albuterol or even plain saline and do more flutter treatments along with extra chest PT to clear that extra mucus out. I also increased my nasal lavage treatments to minimize sinus congestion. Even just getting a little more sleep was beneficial.

“The best way to advocate for your health is to know your body and to be comfortable discussing these issues with your physician.”

These new realizations started to help minimize my getting sick every month. I also tried to avoid eating or drinking too much caffeine just before my period, as this exacerbated lung bleeds by increasing blood flow.

I have also learned that as I have gotten older, hormonal activity changes as well. And a lot happened to my body post-transplant at age 35. First of all, my periods were lasting longer and longer after my transplant. They lasted three weeks instead of one week. Because of that, I became anemic and passed out due to heavy blood loss. I had to go on “The Pill” to regulate my periods. Once on the Pill, my periods became more regular and only lasted a few days – no more anemia. Also, a big change post-transplant was that I became diabetic due

to the medications for immuno-suppression; CF-related diabetes is a common complication of CF treatment and aging with CF. Soon after taking insulin, I noticed another change. In those days just prior to menses, my blood sugars would plummet and I had many low blood sugars. I had to remember to readjust my insulin intake to one or two units less or suffer the lows. Another change after transplant that related to my menstrual cycle was migraines. And they always seemed to correlate with just before my period, after, and some-

times in the middle around ovulation.

Knowing that others experienced hormonal issues, too, made me realize that I had to educate my doctors and try to prevent any new infections that would happen because of my cycle. The best way to advocate for your health is to know your body and to be comfortable discussing these issues with your physician. It is suggested that if you notice a pattern of these or other symptoms recurring at certain times of the month, it would be beneficial to keep a diary of the events. And then bring that diary to a doctor's attention. There may be some ways to lessen period-related symptoms while just being prepared and taking proactive measures for others, like lung exacerbations. Maybe it means having a standing prescription

for oral antibiotics at your pharmacy or a stash of antibiotics at home for emergencies (for lung inflammation and congestion).

Two other issues unrelated to menstruation but equally as common in women with CF are yeast infections and urinary incontinence – both very unpleasant issues to have. Yeast infections can now be cured with over-the-counter creams or pills as well as with anti-fungal agents and/or probiotics that can be found in yogurt or pills at your health food store. Before my transplant, I had a hard time eating yogurt due to the milk content; it made me too congested. I then found acidophilus pills that were probiotic and helped regulate the flora throughout my body to counteract the imbalance caused by taking antibiotics.

Urinary stress incontinence results in women with CF who have a history of frequent and severe coughing. It usually occurs during coughing and/or laughing. Luckily, it can be solved without medication. Before my transplant, this was a common problem for me and I had heard that Kegel exercises could help this, but wasn't exactly sure how to do them nor was I

told to by a doctor. Then I read a small article in *CF Roundtable*, by the late Catharine Martinet, about doing Kegels. She explained how to do it and how it strengthened the vaginal floor to prevent leakage during coughing. Mainly women have this issue but men can also be affected to a lesser degree. Here is a website from the Mayo Clinic for further information: <http://www.mayoclinic.com/health/kegel-exercises/WO00119>. This exercise actually did help, when I remembered to do it. Now, post-transplant, I barely cough so it is no longer a problem for me. This year's scientific CF conference has a couple of panels on this issue and more research is now being done about it.

I have been told that gender differences and symptomology in CF are now being studied. And with more people seeing an adult CF doctor around age 18, many of these issues are dealt with or people are referred to someone who can treat or help them. There is the embarrassment factor: some people will not talk about certain issues with their CF doctors. But I hope that won't be an issue for too long. Most of our doctors know us so

intimately that we should share our other symptoms, which are related to or exacerbate our CF symptoms, as well. Only through education, and that means enlightening our doctors too, can we all benefit.

For some research that has been done, see:

Johannesson M, Lúdvíksdóttir D, Janson C. "Lung function changes in relation to menstrual cycle in females with cystic fibrosis." *Respiratory Medicine* 2000 Nov;94(11):1043-6.
Sims EJ, Green MW, Mehta A. "Decreased lung function in female but not male subjects with established cystic fibrosis-related diabetes." *Diabetes Care*. 2005 Jul;28(7):1581-7.
Miller RJ, Tildesley HD, Wilcox PG, Zhang H, Kreisman SH. "Sex disparities in effects of cystic fibrosis-related diabetes on clinical outcomes: a matched study." *Can Respir J*. 2008 Sep;15(6):291-4. ▲

Andrea is 44 and has CF. She is a Director of USACFA and is the Executive Editor/Webmaster. Her contact information is on page 2.



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Donations over \$10 are tax deductible. Please make checks payable to USACFA, Inc. Send donations to: **USACFA, Inc., P.O. Box 151024, Alexandria, VA 22315-1024**

NEW DIRECTOR

INTRODUCING A NEW DIRECTOR OF USACFA...

Meet Jeanie Hanley

Hello Readers! I've been asked to introduce myself, being one of the two new Directors on the Board of USACFA, and include what you should know about me. Something you may not know is I almost ran over Dustin Hoffman in Beverly Hills (Sorry, Dustin). Thankfully, the job description of Director does not require driving. Actually I'm a pretty good driver, but I live in Los Angeles, so that may have to be put in perspective. I've only had one ticket in the last 10 years, if you must know.



JEANIE HANLEY

But seriously folks, you probably want to know what I can do for you. I have written several articles already for *CF Roundtable*, interspersing a little of my medical knowledge as a physician for 20 years into them. I will continue to do that, as long as I don't run out of ideas (or run over them, whichever comes first). Learning about the latest CF research and advocating for my fellow CFers are also other passions that I hope will be translated to improved medical information and care for you.

What else can I do for you? I will laugh at all of your jokes, even if I've heard them already. Why? Because I love to laugh. Sometimes, much to the chagrin of my children, I will even laugh at my own jokes, but there are some people who tell me it's endearing... Regardless, it's good for my lungs and for yours too. So let's laugh together!

Lastly, you should know that I have nine siblings; against the Mendelian genetic odds, four of us were blessed with CF- all diagnosed as adults. I was 33 years old and already a doctor at diagnosis. Although I initially became a doctor to help find a diagnosis and cure for my sister who was the sickest, it soon became apparent that I needed to find a diagnosis and cure for myself too. Even though my siblings and I had symptoms for many, many years, none of us had elevated sweat chlorides. When genetic testing became available, I finally was able to prove that we had CF.

Unfortunately, my sister passed away nine years later and my lofty goal of curing her could not be met. Being the giving person that she was, I don't doubt that she is orchestrating somehow from her heavenly position, along with other CFers who have passed, the incredible research in progress such as VX-809, Miglustat and many others.

Many thanks to the Board for electing me. It is quite an honor to be part of this outstanding organization. I look forward to working together and hearing from you. Please contact me anytime for more information, to tell me a joke or just to say hello. Peace and Love to you all. ▲

Jeanie is 47 and has CF. She is a new Director of USACFA. Her contact information is on page 2.

INTERNET *continued from page 9*

virus significantly attenuated it, potentially reducing its ability to cause inflammation. But the scientists may need to alter the virus further.

<http://www.sciencedaily.com/releases/2009/07/090720202603.htm>

Israeli Scientist Adapts Antibiotic That May Fight Genetic Disease

A team of researchers in Israel has made a breakthrough in modifying an until-now highly toxic antibiotic so that

it might one day be used to repair defective genes that cause diseases such as cystic fibrosis. Timor Baasov has been able to modify the antibiotic, gentamicin, so as to make it both effective in treating a certain type of genetic mutation and nontoxic enough so that it might be tolerated by humans when ingested on a regular basis. The new compound is called NB54, and Baasov has been able to demonstrate its effectiveness in treating test tube cell cultures

taken from patients with cystic fibrosis. Baasov and his team published their findings online in March in the *Journal of Medicinal Chemistry*. Since then, a team of researchers at the University of Alabama, led by David Bedwell, has had promising results using NB54 in a live mouse. NB54 works by allowing cells to read through a type of mutation called a premature stop mutation, a random piece of scrambled genetic code that stops the cell in the middle of making a

THROUGH THE LOOKING GLASS

Never Say Never

Never expected to get married after so many years
Of trying to find the right fit
Never thought I would find the right person
Who could accept cystic fibrosis, bilateral lung
transplant, diabetes, depression – me
Someone who did not need to have biological
children
Who would settle for the four-legged, canine variety
I feel lucky to have found my mate
It was difficult but he found me
It never would have happened if
Our dogs had not frolicked together in the dog run
They knew a good match and steered us on the
proper path
I am so glad that I waited and never gave up
Never would I have guessed I would be happily
married

– Andrea Eisenman



PHOTO BY DOOLEY

"Through the Looking Glass: Images of Adults with Cystic Fibrosis" and "Caregiver Stories" are projects of Breathing Room, a non-profit organization. Breathing Room hosts these and other projects to facilitate open and candid communication in the CF community, supports the development of a community of adults with CF and provides education and insight for families, caregivers, and medical professionals who impact our lives.

To learn more about us and view more images in the collection, please visit our website at:
<http://www.thebreathingroom.org>

INTERNET *continued from page 16*

healthy protein. NB54 allows the cell to continue and complete the necessary protein. Scientists proved some years ago that gentamicin allows cells to read through stop mutations, but its toxicity remained an issue. Gentamicin is one of a class of highly toxic antibiotics called aminoglycosides that doctors only use as last-resort drugs when all other antibiotics have failed to be effective. Taken in the doses necessary to kill bacteria, gentamicin is tolerable by humans. But

taken on a regular basis over a lifetime, as would be required to treat a chronic genetic disorder, gentamicin could lead to hearing loss, kidney failure and even death. Baasov has found that by stripping gentamicin of its antibacterial properties it remains effective in suppressing stop mutations, but becomes much less toxic for humans.

<http://www.forward.com/articles/112424/>

bioFILM PA™ Antimicrobial

Susceptibility Kit Impacts Outcome in Cystic Fibrosis Patients

For the past two years, the University of Alberta Hospital has been evaluating a new technology from an Alberta biotechnology company, Innovotech Inc. Under the direction of Dr. Robert Rennie and Dr. Neil Brown, bioFILM PA™ has been evaluated in cystic fibrosis patients. Innovotech recognized that current susceptibility test-

Continued on page 33

FROM OUR FAMILY PHOTO ALBUM...



ERIC HYMAN NEAR THE SAN DIEGO COAST.



DEBBIE AND LOUIE AJINI AND THEIR LATE DOG, MAX.



CYNTHIA DUNAFON WITH KATHY AND PAUL RUSSELL LAST SUMMER IN PORTLAND, OREGON.



ISA STENZEL BYRNES, PAUL, KRISTI AND SARAH FELD AND ANA STENZEL, LAST SUMMER IN KANSAS CITY, MISSOURI.



STEVE DOWNEY AND ANDREA EISENMAN CELEBRATE THEIR FIRST WEDDING ANNIVERSARY IN CHICAGO.



LEW AND LAURA TILLMAN AT THE CFRI CONFERENCE.



MICHELLE COMPTON WITH HER BREATHING ROOM IMAGE AT THE CFRI CONFERENCE.



ANA STENZEL AND TRENT WALLACE AT THE CFRI CONFERENCE.



JEANIE AND JOHN HANLEY WITH THEIR WINNING BASKET AT THE CFRI CONFERENCE.



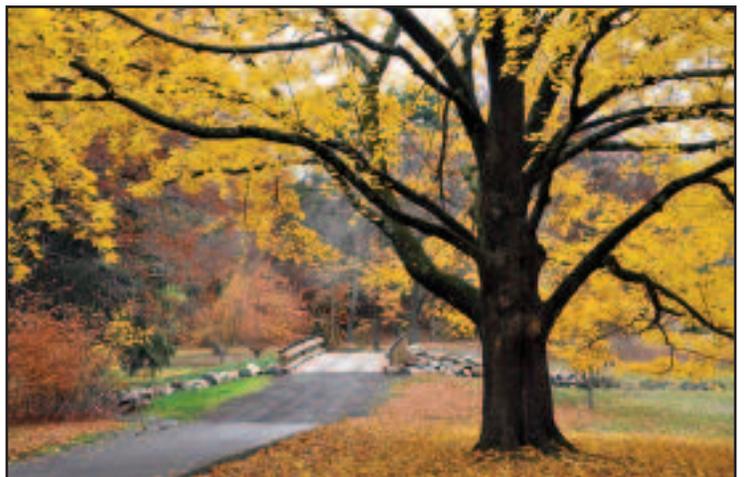
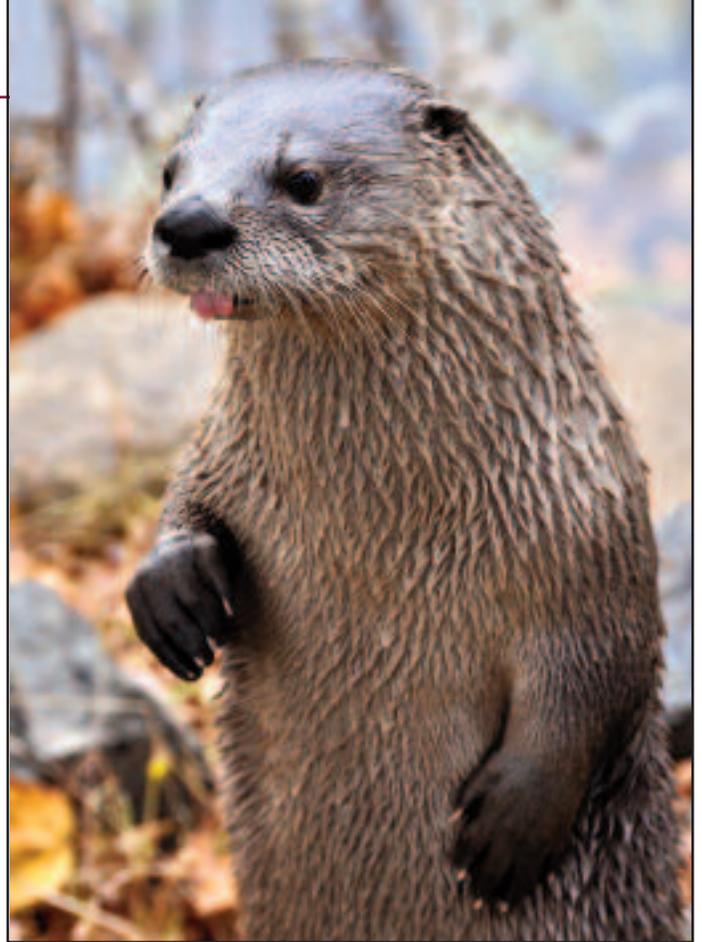
CARROLL JENKINS AND DAVID SOOHOO AT THE CFRI CONFERENCE.

“Fall Back”

By Pammie Post

Hold it. Now what happened to summer? It seems impossible that fall officially begins next week, yet, there is a refreshing crispness in the early morning. Most people around town have been blaming our fleeting summer months on the lousy wet weather. The weather always gets a rotten reputation when it isn't picture perfect. True, we did have an unusual amount of rain but, to my utter delight, the temperatures stayed moderately cooler in comparison to previous years. Our plants loved it. My lungs loved it. Here is my assessment of this summer; time flies faster when life is enjoyed.

One of the sailing highlights of this year was being asked by our Norwalk Yacht Club and the Senior Editor of “Windcheck” Magazine to photograph the First Annual Northeast Beneteau 36.7 Championships Regatta. The event took place on August 22 and 23, a mile off



Greensledge Lighthouse, in Norwalk, CT. Fourteen boats, from as far as away as Annapolis, Maryland participated, including two from our Club. Bill and I used our boat to photograph the races. Due to surrounding storms on shore and on the Long Island Sound, we left after the first three out of five races. Sunday's regatta was canceled due to lack of wind.

Beneteau's are a one design racing class. These boats were all 36.7 feet in length. Like all serious racing boats, when racing, they are extremely uncomfortable, i.e. no cushions in the cockpit, no extra weight allowed, it's bare bones racing. I am not sure if these skippers dumped out their water tanks as well, carrying only a limited amount of water necessary for the crew. It's all about speed.



Going for the Mark

When photographing races, if we are close enough, I like to zoom in to capture action by the marks, start and finish lines. In this shot, Bill positioned us by the windward mark on the course, just out of the way of the racers, so I could photograph the boats coming up to the mark, rounding it and setting their spinnakers for the downwind leg a mile away to the leeward mark.

In this photograph, *Gotcha* just missed colliding with *Mischief*, in the # 3 spot. Though it was a very close call, it was totally exciting. To stop the action, it was necessary to boost the speed to 1/600 of a second. To get all the boats in focus, I increased the depth of field to f/14. Due to the clouds, the ISO was increased to 400.

Camera info: D90 taken on August 22, 2009 on manual settings: 1/600sec., F14, ISO 400, 18-200mm lens at 95mm.

Edie

At times I find unexplored treasures right in our nearby neighborhoods. The Stamford Museum and Nature Center is one of those gems. I discovered this wonderful place last year, having lived 15 minutes from it for 26 years. Hello! Bill had just given me a new camera the day before, a Nikon D90 DSLR, so I, of course, had to test drive it. Where better to do this than at a local Nature Center and one with otters too?

In memory of Edith and Robert Graham, money was given to rebuild the Otter Pond at the Stamford Nature Center. With the help of a landscape architect and others, they built a large pond with current flows, two rock waterfalls, added trees, bushes, grasses, irises, other plants, rocks, wonderful hideaways, and an attached small indoor enclosure. To my sheer delight, this was the best exhibit I have ever seen for otters. Soon after the completion in June 2006, Edie, a North American River otter, arrived as a young pup and has been entertaining

children and adults alike ever since. Robert, or more commonly called “Bert”, joined her in April 2008. Hopes remain high for them to start a family.

Otters are extremely entertaining and a bundle of energy. They love to play; run on land, swim, jump and chase one another around. After meals, this is particularly true for this duo. Edie would chase Bert and then run off and hide, wait and pounce. Edie possesses a captivating personality. She is an extravert with humor. Bert is the opposite, not as aggressive, and certainly quieter. Edie responds well to children. Upon hearing their voices, she gets excited, leaps around, often telescoping or standing up on her hind legs to catch a glimpse of them. But when she smells food, she goes berserk – running, swimming, leaping in mid-air, tracking her keeper with keen eyes and sense of smell – in anticipation of a delicious, small midday snack which could be herring, a portion of tuna, or, yes, raw hamburger bits. Edie was taught early on to stand on a particular flat rock for lunch.

The activity of the otters required a faster shutter speed. To fuzz out the details in background, a smaller aperture (larger lens opening) or F stop helped. Here’s a depth of field (DOF) tip that I read from Corey Hilz’s 2009

book, Focal Digital Camera Guides Nikon D90, “How much will be in focus? Just think small number = a little in focus (f/4, f 5.6) “big number = a lot in focus (f/16, f/22).”

Edie doesn’t always stick out her tongue. Her paws spring into action to catch food. The Nikon D90 passed the test!! I adore this magnificent camera.

Camera info: D90 taken on October 26, 2008 on manual settings: 1/250 sec., F9, ISO 640, 18-200mm lens at 170mm.

Dressed for Fall

In order to catch the best light during daylight, venture out early in the morning or late in the afternoon when the shadows are longer. Overcast days are wonderful too. The sky becomes a natural diffuser – no highlights or shadows. When the sky looks like a blank sheet without detail, try to avoid including too much of it for a more interesting shot. Overcast skies make colors stand out more, perfect for fall foliage shots.

I used a high F stop to gain a greater depth of field, which meant pushing the ISO to 800. In brief, digital cameras use image sensors, which are the equivalent to the film’s former ASA settings. ISO (International Organization for Standardization) refers to how sensitive the image sensor is to light. The higher the ISO, the easier it is to take low light images and or use a faster speed. The ISO combined with aperture and speed, make up a correct exposure. With the Nikon D90, the camera can easily afford a high ISO of 400-800 without showing a grainy effect, or in digital terms, “noise”. Kodak’s popular black and white Tri-X film 400 ASA was/is recommended for sports and low light photography. The film is grainy. Among many things that digital offers over film is the ability to change the ISO for each picture. With film, there isn’t that option; well, there is a way to do it with rewinding and swapping films etc., but it is risky.

Continued on page 32

Call to All Artists

If you wish to submit art that expresses your feelings about CF or anything on your mind, please send photographs of any media: paintings, illustrations, collages, drawings, sculpture, etc. to:

cfroundtable@usacfa.org. or you may mail them to:

USACFA
PO Box 1618
Gresham, OR 97030-0519.
Please include your name and contact information.

INTRODUCING A NEW DIRECTOR OF USACFA...

Meet Julie Desch

Hi. I'm Julie Desch, and I am honored to serve on the USACFA Board of Directors. I am 48-years-old, and carry two copies of that nasty delta F508 gene. I was diagnosed as a baby, after two of my five older siblings were found to have CF. Sadly, they have both since died, and I am on my own carrying the CF baton for the Desch family.

I currently divide my time between taking care of myself, my two kids, my six dogs (I know, I know), my partner—well, really she takes care of me, and running my own non-profit corporation, New Day Wellness. In a former life, I went to medical school at Stanford University and studied and practiced surgical pathology. There, I was privileged to work in the CF Research Lab with Dr. Jeff Wine. Recently, I've been having fun going back there to help out occasionally. Nothing has changed.

Through New Day Wellness, I

coach wellness to people with chronic illness (mostly CF). I am a fitness fanatic, which I firmly believe is why I am still here to write this, and I like to share my passion with others. Wellness coaching is a fairly new field, and I feel lucky to have discovered it after I realized practicing medicine was killing me.

I do a fair amount of volunteer CF work... I've been a clinical trial victim, I mean subject in numerous studies. I am on the Research Advisory Board for CFRI, a Patient/Parent Advisory Board for my clinic, and I am helping a few CF centers start up exercise programs for their patients.

I also love to write, which I've been doing for the *CF Roundtable* for a few years. I write the Wellness Column where I wax on about all things "wellness." I also write a blog where I talk about living happily in bodies that don't work so well. It is appropriately found at www.sickandhappy.com. ▲



JULIE DESCH

DESCH *continued from page 1*

where you have to fill in the answers by coloring in ovals on a separate sheet...all the answers after the one you forgot are wrong...chaos ensues). No CFTR protein...pretty severe CF.

A Class II mutation is one where the gene codes for a protein that is constructed by the cell machinery, but because of the error from an amino acid deletion in the gene, the processing of the resultant protein is messed up. As a result, the protein is defective in folding, stability, and channel gating (the opening for chloride ions is not regulated properly). Because it is unstable, not much of it makes it up to where it is needed at the cell membrane. Our friend, delta F508 is a Class II mutation.

Class III mutations allow for the gene to code for a CFTR protein which makes it up to the membrane, but as a result of this "milder" mutation, the CFTR channel is not regulated or activated properly. G551D is an example.

Class IV mutations are similar to Class III in that a protein is made and gets up to the surface of the cell, but it has "altered conductance." The ion channel just doesn't work as well as it should. R117H is an example.

Finally, Class V mutations are those where there is simply reduced synthesis of the CFTR protein.

Clinical Trials 101

You often read or hear about newly

developed drugs being tested on humans in "clinical trials." These trials occur in a series of steps, or phases, that are designed to answer different questions.

Phase I trials are when researchers test a new drug in a small group of people for the first time. These studies evaluate overall safety of the drug, look to find effective dose ranges, and document any side effects.

Phase II trials are designed to **evaluate effectiveness** of the drug and are generally performed with a much larger group of people. Safety continues to be monitored closely.

Phase III trials are done with very large groups of people to **confirm effectiveness**, monitor side effects, compare it to commonly used treatments, and

collect information that will allow the drug to be approved for use.

Now, back to regular programming:

When gene therapy was not proving to be wildly successful, some companies started to ask if the defective protein could be fixed. Fortunately, a technique known as “high-throughput screening” was being developed just as the need to find ways to tweak the CFTR protein was becoming glaringly apparent. Very simply, high-throughput screening uses automation (robotics and high-speed data processing and control software) to rapidly test hun-

“A technique known as “high-throughput screening” was being developed just as the need to find ways to tweak the CFTR protein was becoming glaringly apparent.”

dreds of candidate “small molecules” to find the ones that show a specific biological activity. In the case of CF, they were looking for molecules that could assist with translation of the RNA “message” to form a CFTR protein normally, or molecules that could assist CFTR in getting up to the membrane, or molecules that could open the dang thing up and let the chloride ions flow as they should.

One company, PTC Therapeutics, found a compound called PTC124, which could “read through” the STOP sign on the Class I nonsense CF mutations. PTC124 (now called Ataluren) only works for Class 1 (nonsense) mutations, of course, but clinical studies so far are looking very promising. Phase I and II studies have confirmed that Ataluren is safe, orally tolerated, and showed encouraging efficacy. A much larger and long-term Phase III trial has been scheduled.

In the case of CFTR protein mod-

ulation, Vertex Pharmaceuticals looks for small molecule correctors and **potentiators**. Simply put, a corrector gets the CFTR protein to the membrane in larger numbers. This would be helpful in the Class II CF mutations such as delta F508. A potentiator works on the protein already at the membrane, increasing its effectiveness. This kind of drug could potentially be beneficial in several of the mutation classes.

VX-770, an investigational CFTR potentiator, is intended to increase chloride ion transport through the defective CFTR protein. Vertex chose to specifically look at people with the

Class III G551D mutation in the early phase trials of VX-770 because, in this mutation, the protein is already where it needs to be on the membrane. It just needs to be tweaked to open properly. They figured that although only 4% of people with CF carry this mutation, the odds of showing effectiveness would be best in this small group of patients.

And, indeed, they were right! Not only did Phase II trials show a marked (10%) improvement in lung function after only two weeks of treatment, they also showed that both nasal potential difference (PD) and sweat chloride levels moved distinctly toward normalized values (this is exciting because no treatment ever has shown to change the sweat chloride levels). Importantly, when people stopped taking the drug, lung function values, sweat chloride values and nasal PD values returned to their baseline values.

Based on these positive results,

Vertex is now initiating larger, Phase III trials. These are designed to look at larger numbers of children and adults with the G551D mutation over a longer period of time. In addition, a Phase II study of VX-770 in patients with CF aged 12 years and older who are homozygous for delta F508 is planned to start in the third quarter of 2009. The hope is that VX-770 will measurably increase the effectiveness of the small amount of CFTR protein that actually makes it to the membrane in delta F508 CF. If so, then all we need is a **corrector** to get more of the protein to the membrane, and throw in a dash of VX-770 to create a “Vertex-cocktail” of sorts.

Vertex is hoping that VX-809 is just that corrector (and so am I). This molecule is designed to increase the amount of deltaF508 CFTR protein on the surface of cells lining the airway. It is one phase behind VX-770. So far, Phase I studies have not shown any safety or tolerability issues. A Phase II study of this drug is now underway. Where can I sign up?

In summary, I think the message is this: There is serious cause for hope that one day soon, we will take yet *another* daily pill (or two...) that is going to improve our lives beyond anything that has yet been discovered. Is it going to “cure” CF? Not likely. A scarred pancreas is not suddenly going to produce enzymes or insulin. Damaged lung tissue is still damaged. I am not suddenly going to have a normal FEV₁. But if I knew that a daily pill might slow or even halt the downward slide of lung function that has up until now seemed inevitable...I’d be pretty psyched! I might even volunteer to write an article about it. I have only one suggestion for Vertex Pharmaceuticals. Will you please give these things proper names? ▲

Julie is 48 and has CF. She is a physician and is a new Director of USACFA. Her contact information is on page 2.



UNPLUGGED...

With Brendan and Devin

By Richard De Nagel

Hello, and welcome to another exciting edition of “Unplugged”, which promises to be especially interesting. I have interviewed not one, but *two* people, for this issue. You may be thinking that I interviewed siblings who both have CF, or a parent and their adult child with CF. In fact, you will meet two brothers; one with CF and one without.

As I was speaking to one of them about CF, thinking he had it, I found out that he did not. As we discussed what that was like for him, it brought to mind the extent to which our partners, parents, and siblings are affected by this disease. Some days I forget, and take for granted all the people in my life who are affected or inconvenienced by CF. It’s a good reminder that we are not the only ones who have to deal with our disease.

This past summer I was at the Cystic Fibrosis Research, Inc. (CFRI) Conference. It offers a great opportunity to bring together people with CF, doctors, parents of kids with CF, nurses, and friends. We learn about the latest CF research, get lots of good products from drug reps, and enjoy hanging out with other CFers.

While in the hotel lobby one evening, I saw a young guy with a blue dot on his name-tag – an indicator that he has CF. We started chatting, and I learned a lot about his family and his brother with CF. He came to the conference to hear lectures and learn more about CF, and to eat some food. Our conversation turned from a casual chat into a very interesting talk about CF and all of its various effects. So, I’d like you to meet Brendan, and his brother, Devin, who is a person with CF (PWCF).

1) Name: Brendan Wakefield



**THE WAKEFIELD FAMILY:
SCOTT, KATHLEEN, DEVIN
AND BRENDAN.**

2) Age: 22

3) Where do you live? Santa Cruz, California

4) When were you diagnosed with CF?

My brother was diagnosed when I was three years old.

5) Who is your doctor? Hospital? Do you like him/her?

Devin has several doctors, I’m not sure who is primary, and he mostly goes to Stanford Hospital. He’s now making the transition from Children’s to the adult hospital.

6) How would you describe your health now?

I’m staying fairly healthy; I just got over a severe case of Mono. Devin was just discharged from Packard Children’s Hospital, after a run of IVs, to get him into top shape before going off to Santa Clara University.

7) Do you believe in a Higher Power? Are you religious?

I’m still trying to figure out my solid beliefs in this world. Maybe that’s what I’ll say my whole life, if I can’t make up my mind. I’m a very spiritual person and believe in some kind of energy humans cannot fully

understand. Maybe that’s what you call God or Love.

8) What are your hobbies? Does CF interfere?

I have too many to list here, but my latest interest is kitesurfing. It’s like windsurfing, but you’re flying a giant parasail above you. I want to take Devin out surfing because it’s basically one long sinus flush, but I have yet to get him down to Santa Cruz. In high school he and I were both really into soccer, so we would go on runs and play in the park together to get Devin to cough up extra mucus.

9) What is your most embarrassing CF moment?

At the most recent CFRI conference in the Bay Area, I wore my brother’s ID badge because he couldn’t make it, and I wanted to use his ticket to get free food. I forgot that his badge had a blue dot on it, indicating that he was a CF patient. CF patients aren’t allowed to serve themselves, so I made some people a little annoyed as I shoveled tons of food onto my plate.

10) What gets you through the tough days?

I help Devin through the toughest situations by trying to lessen the intensity of everything. I like to joke and play video games, anything that will distract him. Sure, there are times when he needs to focus, but that’s Mom’s job. I like to think that I’m there to support Devin and then have fun with him.

11) What do you hate most about CF?

Most of all, I hate how much CF takes from Devin’s life. A major percentage of his time and attention is spent only on the procedures and

tasks CF requires him to complete. This means twice as many bags of luggage to go on a family trip, or a two-hour treatment before he can start his day. Our family must have a ton of energy.

12) Do you have kids? Want them?

I am nowhere near the time in my life to think about kids, but right now I like the idea of adoption.

13) What do you look forward to?

I look forward to getting my book finally published and not having the pressure of tenure on my back. Even though I'm scared sh*tless, I'm looking forward to a potential career in Psychology...while I kitesurf, mountain bike, surf, skate, and snowboard along the way.

14) Do you think having CF is a good thing or a bad thing?

If there's one answer to that question, CF is a bad thing. But that doesn't mean there aren't good life lessons with it. To deal with such an impact on one's life or one's family is a tremendous feat, and I think it's something to be proud of. I also think that being so closely exposed to death or the potential of death has given our family a perspective on the fragility of life most other family's do not endure.

15) What is your favorite color?

Sunfire yellow. For those of you who don't have the GM Auto Color Guide memorized, it's a shade between yellow and orange.

16) Do you spend time with other people who have CF? If so, what do you do, and how important is this to you?

I actually do not spend much time with other people with CF. The only time I do is at the CFRI conference. I think this actually gives me a limited perspective on the range of CF. Most of what I know about it is what Devin experiences. Of course, I've heard about other people's situations in the CF community, but that's different from truly knowing what's out there.

17) Do you spend time educating yourself about CF? How important is this to you? What effect does this have on your treatments? Rapport with your doctors? Self-image?

I probably don't spend as much time educating myself about CF as I should. All my life it's pretty much been up to Devin and my parents to take care of the treatments, medicines, and hospitalizations. When Devin is on home IVs I help out and know what needs to be done, but I sometimes wonder if I should know



DEVIN WAKEFIELD

more for emergency situations.

Now, here is Devin:

1) Name: Devin Wakefield

2) Age: 18

3) Where do you live? Palo Alto, California, USA, Earth.

4) When were you diagnosed with CF?

Within a few months after birth. I'm not entirely sure when, exactly.

5) Who is your doctor? Hospital? Do you like him/her?

My doctor is Dr. Moss. He works at the Packard Children's Hospital. I do like him. He's been very good to me and always offers the best advice I could hope for. He also has very nice staff.

6) How would you describe your health now?

Well, right now it is under improvement. I'm on IV antibiotics and yesterday my Pulmonary Function Tests (PFTs) went up from

before I started. So, hopefully I'll have gotten back to my baseline.

7) What is the newest music in your iPod/CD player?

Hmmm, apparently it is a song called "The Lalala Girl". It's a nice techno song.

8) What is your favorite music in your iPod/CD player?

That's a good question. I'm not sure what I'd call my favorite, but it is probably a techno song. Maybe this one called "Di Di Di", by Basshunter, who is a Swedish DJ.

9) Are you working? How are you doing with that?

I'm not working for pay, but I will be going to college soon; so I guess you could consider that work.

10) Do you believe in a Higher Power? Are you religious?

I do. I consider myself to be Roman Catholic. I'm not really *that* religious or anything, but I do have a great Saint Anthony prayer that works very well, and I pray occasionally.

11) What are your hobbies? Does CF interfere?

I love to play soccer, mess around with computers/websites, read books and newspapers, play computer games, and other related activities. CF only really interferes if I need to be admitted to the hospital, but even then I can connect to the internet (Except one game I like to play has issues when I try to play it. I'm not sure what that is about).

12) What is your relationship status? Happy about that? Does CF interfere?

I'm single. I wouldn't say I'm too happy about it, but I haven't really had my heart broken yet, so I suppose I'm somewhat carefree. And CF hasn't really had anything to interfere with, so not yet.

13) What is your most embarrassing CF moment?

I don't know. I don't think I

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WELLNESS

Sex Hormones: How They Play A Role in CF Health

By Julie Desch, MD

Reviewing

Sex Hormones: How They Play A Role in CF Health

Presented at the CFRI Conference

By Marcia Katz, M.D., Associate Professor, Medicine-Pulmonary

Baylor College of Medicine, Houston, Texas

The focus of this *CF Roundtable* is “Gender-related Problems in CF”, and as luck would have it, my assignment for this year’s annual CFRI conference was to cover the talk given by Dr. Marcia Katz on none other than, sex. Okay, not really sex exactly, but I got your attention, didn’t I? The upside is that I can combine my Wellness column with my review of Dr. Katz’s talk.

Dr. Katz actually discussed “Sex Hormones: How They Play A Role in CF Health.” It was a very interesting discussion of how sex hormones relate to our health in ways that we probably haven’t thought of before. At least I hadn’t.

But first, a little review of known gender differences in people with CF. Cutting right to the chase, the big difference is that, historically, there has been an increased mortality reported for women with CF when compared to men. The bulk of studies over the past two decades have shown that women with CF have a worse prognosis overall; they participate less in aerobic exercise, ingest fewer calories, perform less physical therapy, exhibit an accelerated decline in FEV₁ with acquisition of *Pseudomonas aeruginosa*, and show increased asthma reactivity. While this has been undisputed, it has never been adequately explained. Though this is sobering for

those of us who lack a Y chromosome, the good news is that as more attention is given to aggressive therapy, this sex-related difference appears to be dissipating.

But as Dr. Katz pointed out, women tend to be more vulnerable than men to numerous inflammatory diseases in addition to CF. Asthma, sarcoidosis, idiopathic bronchiectasis, several connective tissue disorders such as lupus and rheumatoid arthritis, and my favorite (only because I can actually pronounce it) lymphangioleiomyomatosis (LAM) are all more common in women, and women tend to do more poorly than men. What is common in these disorders is that inflammation runs amuck, and women clearly bear the brunt of this more than men.

Because of this, it has always been suspected that estrogens play a significant role in these conditions. Now, I don’t really want to go into a biology class here, but I sort of have to. During the menstrual cycle, estrogen and progesterone do a little two-step. Estrogen slowly rises and peaks right before ovulation (follicular phase). Ovulation occurs after estrogen does a nosedive, and then progesterone starts to slowly rise (luteal phase). After about 14 days post-ovulation, both estrogen and progesterone are very low, and menses occurs. I know that was painful and I apologize, but it had to be done.

Moving on, generally speaking, estrogens are pro-inflammatory, which might at least partly explain the increased prevalence and severity in the above-mentioned conditions. In addition, Katz pointed out that progesterone and testosterone (yes, women have a tiny bit) relax smooth muscle. As evidence of this, FEV₁

tends to be higher in the second half of the menstrual cycle (luteal phase), when progesterone is high.

It would be hard to write a nerdy article like this about CF without mentioning mucus. In this case, however, this is where it gets interesting. It turns out that the CFTR protein that lines the respiratory tract (and other epithelial surfaces) is not the only kid on the block when it comes to moving chloride ions. There is another way for chloride to escape the cell, and that is through a calcium-(Ca⁺) regulated chloride channel (CaCC). This channel is independent of CFTR but responsive to intracellular calcium concentration. When Ca⁺ goes up inside the cell, the channel opens, and chloride can go out.

Here’s the kicker: a recent article by Coakley et al. reports that elevations in the major estrogen hormone in humans reduce Ca²⁺-activated Cl⁻ secretion by airway epithelial cells in culture, and this disrupts ion and water balance. They also showed a similar measured decrease of nasal epithelial Ca²⁺-activated Cl⁻ secretion in women with CF during the menstrual cycle phase at which estrogen is at its highest. As a result of these findings, the authors suggest that, “for about one week, of a four-week menstrual cycle, women with CF will have a reduced ability to efficiently clear airway secretions, the buildup of which is a hallmark of CF.” Can you believe that? First, the CFTR doesn’t work for beans. Then on top of that, we have to deal with this?

All right, then, so life isn’t fair. What to do about this? Well, it’s too early to make any conclusions, but it is interesting food for thought. The authors suggest that these data warrant

the testing of an alternative avenue for CF therapeutic development. Now, don't all of you women with CF go out and get Tamoxifen! It's way too early for that! One thing you CAN do without any chance of harm is to up your respiratory clearance techniques, exercise and maybe even hypertonic saline during that one week when estrogen is peaking (roughly 7-14 days after the first day of your period). Hey, it's only one week!

I might point out for those of us who are menopausal or perimenopausal, this is yet one more reason to celebrate!

Now, for the males in the crowd: It is known that serum testosterone levels are frequently low in adolescent and, sometimes, adult male patients with CF. To sum up Dr. Katz's message to boys and men with CF, low testosterone is not to be sneezed at! Along with the obvious physical concerns

such as delay in pubertal onset, bone density, and body composition issues, low testosterone can lead to decreased libido, decreased energy, mood changes, and frank depression. As you might imagine, depression is much more common in CF than in normal populations (studies have ranged from 29% to 46% in CF adults vs. 13% in the "normal" population – note the use of quotation marks). Though less marked, there is also an increase in adolescent depression in CF vs. normal. Low testosterone might explain some of this difference in males.

The good news is that low testosterone is treatable! Easily. It just needs to be considered in the differential diagnosis of depression and low energy in men and adolescent boys with CF. In Dr. Katz's experience, simple testosterone replacement therapy has made a world of difference to some of her male patients. Now, similar to the

Tamoxifen warning, all of you men and boys shouldn't just go out and score some testosterone on the streets (can you do that?!). Instead, have a candid conversation with your CF doc, letting him/her know if you experience low libido, low energy, low moods, etc., and tell them you are wondering if your testosterone is low. It's an easy blood draw, and an easy fix (compared to some of our other "fixes").

All of the talks at this year's conference were great, and this one was no exception. My favorite thing about Dr. Katz (besides the fact that she is extremely knowledgeable and quite nice) is that I AM TALLER THAN SHE IS!!! That never happens. ▲

Julie is 48, has CF and is a physician. She is a new Director of USACFA. You may direct your questions regarding CF-related health issues to her at: jdesch@usacfa.org.

One of USACFA's Angels – Mike Mittelstead

By Kathy Russell

In July 1990, Connie Knoles (a founder of USACFA) and I went to many print shops in the greater Portland, Oregon area, looking for a good one to produce *CF Roundtable*. We knew very little about producing a newsletter and we wanted to find a shop that would help us through the process. Another member of our adult support group had called every printer in the Portland phone book trying to find a few that sounded like they could help us. She gave us a list of half a dozen shops.

We visited each shop and explained our situation. We found some shops that almost scared us with their lack of grasp of our situation. When we arrived at the

Metropolitan Printing shop, we were pleasantly surprised by what we found. We were introduced to Mike Mittelstead, who subsequently became a large part of producing *CF Roundtable* for USACFA.

Mike has been our mentor, guide and angel for 19 years. He took the "flak" from the people in production who complained about our lack of skills in the early days. He never has complained to us, when we brought in incomplete files or awfully dim photos. Instead, he explained how we could do things better. He has made constructive suggestions all along the way.

He has worked to keep our costs as low as possible. He has given us some of the things that we needed, such as notepads and business cards, at no cost

to us. Other people at MetroPrint have come and gone (even the owner has changed) but Mike has been a constant for us.

Every issue of *CF Roundtable* is read cover-to-cover by Mike and he catches the typos that our proofreaders sometimes miss. He has met several of our Directors, through the years, and he has come to know all of us through *CF Roundtable*. He celebrates our accomplishments and mourns our losses, right along with us. Even though he doesn't have CF, he seems to "get" what it means to have it.

We at USACFA are very grateful to Mike for his 19 years of helping us to produce a newsletter that we can be proud of. Thanks for being our friend, Mike. ▲

A Swedish Care Model: What We Can Learn

*Presented at the CFRI Conference
By Birgitta Strandvik, M.D., Ph.D.,
Department of Pediatrics
Institute of the Health of Women
and Children
Göteborg University
Göteborg, Sweden*

Reviewed by Cynthia Dunafon

CFRI invited Dr. Birgitta Strandvik, a clinician and researcher, to speak about the CF care regimen in Sweden. Her talk began by comparing the Swedish CF population with the US CF population. Although different in size, they share roughly the same median age, BMI and distribution of the $\Delta F508$ mutation. Yet Sweden's approaches to antibiotic use, airway clearance, and nutrition reveal how people with CF can thrive under radically different treatment regimens.

Antibiotics

Swedish CF patients begin IV antibiotics at the first *clinical* sign of infection. At the end of the 7-14 day treatment, they rarely transition to long-term antibiotic therapy such as TOBI or Colistin. As a result, Sweden uses IV antibiotics more frequently than the US but far fewer antibiotics overall.

Inhaled anti-pseudomonal drugs also are used less frequently because clinicians treat *Staphylococcus aureus* infections more aggressively than *Pseudomonas aeruginosa* over the long term. Oral Flucloxacillin is the antibiotic of choice for Staph, which does more damage to the lung tissue (an opinion shared by many European clinicians). For Strandvik, the issue is not whether to use antibiotics, but how to use them. Of note: both MRSA and mucoid *Pseudomonas* are rarely seen in Sweden.

Airway Clearance

The audience sat up and took notice when Dr. Strandvik described airway clearance techniques used by Sweden's four CF centers. For most patients, physical activity and forced expiratory techniques such as auto-genic drainage take the place of chest percussion – both hands-on and machine-assisted. So no CPT, no Vest and 70% of the Swedish CF population exercise regularly. Not only does this lighten the treatment burden of CF care but even more important, it shifts “therapy” to activities that are more enjoyable and conducive to overall physical health – an important factor if one plans on living into old age.

Pulmozyme and hypertonic saline are available in Sweden, but not widely used. Instead, Swedish clinicians prescribe two drugs to help break down mucus in the body: Bromhexine (taken orally or intravenously) and N-acetylcysteine (taken via inhalation), which older CFers will recognize as Mucomyst. Bromhexine (Bisolvan) and Ambroxol (Mucosolvan) are derivatives of vasicine, an alkaloid found in the leaves of the *Adhatoda vasica* plant common in Nepal, India and Pakistan. Both have mucolytic and anti-inflammatory properties. Although unavailable in the US, Bromhexine has been used in the Swedish CF community since the 1980s. This inexpensive drug thins mucus in CF lungs and sinuses. N-acetylcysteine (NAC) enhances the anti-inflammatory response in the cells and thins mucus by breaking disulfide bonds in mucins, a type of glycoprotein secreted in the mucus glands in the respiratory tract. According to Dr. Strandvik, N-acetylcysteine and Bromhexine work *together* to cut the disulfide bonds con-

tributing to mucus viscosity.¹

Are these methods of airway clearance successful? Although median FEV₁ values are not markedly different between the two countries, a difference does appear when we look at levels of lung function in the two adult populations. In Sweden, 30% of CF adults have completely normal lung function, while only 16% of CF adults in the US can say the same. Ten percent of Swedish CF adults have very low lung function, while 20% of US adults fall into this category.

Nutrition

Since the 1960s researchers have noted abnormal levels of essential fatty acids in people with CF. In Sweden, people with CF are encouraged to add essential fatty acids to their diet (via corn oil or sunflower oil mixed in food) to help correct this imbalance. When they do this, most CFers eat a normal diet and do not require supplementary meals/snacks to maintain their energy. But adding oils to the diet is only one part of the picture of essential fatty acids in CF. Dr. Strandvik and her colleagues have found that essential fatty acid deficiency may not just be a secondary result of pancreatic insufficiency but also a primary result of the CFTR gene's impact on cellular metabolism. Essential fatty acids and their derivative signaling molecules (called eicosanoids) influence several cellular mechanisms including: calcium release, ion channels, gene expression and phospholipase activation. If this is indeed the case, we should see a relationship between these acids and CF symptoms.

And we do. Here is one simplified example of how researchers are probing this relationship. (Any errors contained herein are mine.) People with CF tend to have an abnormal ratio of two different essential fatty acids: high levels of arachidonic acid (AA) and lower levels of docosahexaenoic

acid (DHA). AA is a precursor of pro-inflammatory mediators in the cell, and DHA functions as an anti-inflammatory mediator. Our cells need both substances, but only the right combination of these two fatty acids will enable them to keep the process of inflammation in check. Inflammation is one factor contributing to the progression of CF airway disease.

So how do these fatty acid levels get out of synch? When the body needs extra energy, the fatty acids in the cell will metabolize quickly into a cascade of new acids, depriving the cell of early-stage fatty acids it needs for other functions, such as fighting inflammation. The ratio of AA to DHA discussed above, stems, in part, from the metabolic rate of the fatty acids. The fatty acid Linoleic Acid (LA) metabolizes into pro-inflammatory AA. When the metabolic rate is high, too much AA is produced and too much LA has been used up in the process, leaving the level of LA in the

cell lower than it should be. The production of anti-inflammatory DHA depends on a certain level of LA in the cell, so low levels of LA skew the ratio of AA to DHA even further in favor of inflammation. Dr. Strandvik has shown that lower levels of Linoleic Acid (LA) have been correlated with lower FEV1 readings in CF children. In the Swedish care model, Intralipid is given to CF patients whose level of Linoleic acid is too low.

The jury is still out on what therapeutic application is best for the CF population (especially since fatty acid levels appear to be genotype specific).

Conclusion

I am doing a Vest treatment while I type this article, and I have not packed my bags for Sweden - yet. Nevertheless, this presentation on Swedish care has pushed me to reconsider what is necessary in my CF regimen. The hard work of adapting affordable treatment strategies to individual needs – social, personal and

financial – is more important than any particular drug or device. Having been involved with CF patients since the 1960s, Dr. Strandvik's approach to quality CF care reflects a long familiarity with ever-changing treatment strategies and her keen insight that innovative treatment doesn't just mean reaching for what is new, but rather selecting what is best from the old and the new.

¹Interestingly, CF researchers looking at glutathione deficiency in the CF airway are revisiting NAC. Inflammatory airway neutrophils release too many oxidants in the lung. When taken orally, NAC is transformed into glutathione, which might help decrease neutrophilic inflammation in the CF lung. (See Tirouvanziam, 2006.) ▲

Cynthia is 45 and has CF. She is a Director of USACFA and is the Vice President. Her contact information is on page 2.

CFRI REPORT

An Overview of the 2009 CFRI Conference

By Jeanie Hanley

The CFRI Conference took place on July 31 to August 2, 2009, at the breezy and beautiful Sofitel Hotel in Redwood City, California. From start to finish, the conference was outstanding, especially for my husband and me - as newcomers.

Prior to the official start of the conference, Isa Stenzel-Byrnes welcomed all new attendees. Among the many important messages was the prevention of cross-infection. I had been very curious about this and quickly learned about the “three-foot rule”,

“elbow hugs,” blue dots on badges to recognize those with CF, having a non-CF person sitting in between two CFers at tables, and the plethora of available face masks, hand sanitizers, and individual water bottles.

It soon became clear that the hygiene guidelines would be drummed into our heads at every turn by fellow conference attendees, organizers and, particularly, by Andrew Byrnes, the well-spoken and charming conference emcee who is quite funny and should seriously think about performing comedy regularly.

The atmosphere was constantly

energized with all sorts who had a tie to CF – adults, parents, significant others, friends, medical personnel, sponsors and exhibitors, etc. Most were getting reacquainted or newly acquainted. I was very excited to finally meet Ana and Isa, whose book, “The Power of Two”, was such an inspiration.

From the start on Friday evening to the finish Sunday afternoon, the conference was jam-packed with events – excellent speakers, sharing meals, the banquet with prizes and raffle, support group sessions, CFRI Executive Director Carol Jenkins pulling in others to dance, “Winners’ Circle” Hospitality Suite and much more.

You’ll hear more about the very interesting talks and speakers in this *CF Roundtable* issue, so not much

Continued on page 31

New Findings to Address Tough Psychosocial Challenges in CF

*Presented at the CFRI Conference
By Alexandra Quittner, Ph.D.,
Professor of Psychology,
Pediatrics, Otolaryngology
University of Miami
Coral Gables, Florida
Reviewed by Laura Tillman*

Do you find living with CF to be a bit challenging at times? You know - the daily routines that take up hours of your time, the trips to clinic and the hospital, the ups and downs in how you feel from day to day and even hour to hour, the social isolation from others who experience the same and, perhaps, even some depression and anxiety as a result of all of this? Well, you're not alone. Although those of us living with CF (and that includes caregivers) deal with this every waking (and non-waking) moment, it doesn't seem to be reality until the researchers investigate what we already know in order to give it validation! And that's just what Alexandra Quittner set out to do in her presentation - to make it all reality by discussing the psychosocial puzzle of living with CF - which consists of Adult Roles, Depression and Anxiety, Quality of Life, and Overcoming Barriers. These four puzzle pieces were illustrated by citing the results of a new national survey, as well as discussing screening for depression/anxiety, and introducing research aimed at identifying barriers to adherence as well as new interventions for overcoming those obstacles.

"The Adult Data for Understanding Lifestyle & Transitions Survey" indicated that since CF patients are living longer they are:

1. Pursuing adult roles in life.
2. Living independently, which is

a key step in assuming adult roles.

3. Attending college, technical school, and in serious relationships/marriage.

4. Most likely to disclose their condition to relatives and close friends rather than to employers, colleagues, acquaintances, and neighbors.

The fact that adolescents and adults with CF are more likely to become depressed than the general population and that few studies have examined the effect of depression on

“While adherence may alleviate, to a degree, some physical symptoms, it doesn't counter the feelings of social isolation.”

health problems was another issue that Dr. Quittner examined. She presented results from a survey of 78 people that indicated that even low levels of depressive symptoms should be acknowledged and dealt with, since depressive features help to identify those at risk for poor adherence, more pulmonary exacerbations, and a poorer quality of life. Additionally, caregiver adherence (as in giving enzymes to his/her child with CF) was also diminished if the caregiver was showing signs of depression.

Dr. Quittner then continued to illustrate the consequences of poor adherence to prescribed medical regi-

mens. These include increased symptoms and complications leading to more hospitalizations and possibly greater lung damage, higher family stress and conflict, and rising health care costs. Adherence is impacted by one's knowledge and skill level, ability to communicate with the health care team, and developmental issues. She proposed that the clinic team has the responsibility to provide strategies to overcome adherence issues and explored two new interventions that are currently being utilized: "iCare Study" and "CF My Way". Dr. Quittner then presented some anecdotal findings which indicated that these measures may have positive results in overcoming barriers to adherence, if the CF clinic team is able to institute them.

Dr. Quittner's four disparate puzzle pieces mentioned above were put together with the key piece - adherence. While it sounds simple, I'm not sure that I agree that adherence is the single solution to the psychosocial problems faced by those with CF - or any chronic illness. I think that there are many other factors that need to be addressed, in and of themselves. While adherence may alleviate, to a degree, some physical symptoms, thus decreasing some anxiety or depression, it doesn't counter the feelings of social isolation or other concerns that may also be contributing to one's depression or anxiety. I could point out other areas that leave me somewhat skeptical and perplexed as to how adherence will solve them, but I will let you, the reader, puzzle over that! ▲

Laura is 61 and has CF. She is a Director of USACFA and is the President. Her contact information is on page 2.

Panel Discussion: Having A Career When You Have CF

*Presented at the CFRI Conference
By Rich DeNagel, Teacher; Tom Martin, Mechanical Engineer; Jeanie Hanley, Physician; Everett Rice, Legislative Coordinator; Dan Lagasse, Pastor; Anabel Stenzel, Genetic Counselor
Reviewed by Maggie Sheehan*

The last time I wrote, I talked about not knowing what kind of career I wanted once I graduate from college. I am only eight months away from that milestone and still am not so sure in what direction I want to go. I did, however, get a little better insight into my decision-making process from the panel of experts at the recent CFRI conference.

The CFRI “Career Panel: Making it Work”, featured a group of six adults who have, or have had, diverse careers as varied as their health regimens. The key message I took away was what works for one person, does not work for everyone. The panel’s work experience included a high school history teacher, doctor, legislative coordinator, pastor, engineer, and genetic counselor. Some were currently employed while others

were just getting back into their professions after being on disability for a period of time.

It was comforting for me to know that all of these people have struggled in their personal and professional lives and

back into teaching full-time and just started his second year this September.

Many on the panel discussed their time on disability and how that affects one’s mental health. So often we associate our identity with how much we



L TO R, TOM MARTIN, ANA STENZEL, EVERETT RICE, RICH DENAGEL, JEANIE HANLEY AND DAN LAGASSE.

that making decisions about a lifelong career is not easy or set in stone.

Rich DeNagel was a perfect example of this. Many of you know Rich through his “Unplugged” column in *CF Roundtable*. I learned even more about his struggles and accomplishments through listening to him speak on the panel. Rich talked about being a teacher and how, after a couple of years, he needed to take a step back and focus on his health. To do this, Rich had to go on disability. Last year Rich went

contribute to society. Rich found volunteering a good way to keep busy and fulfilled.

This panel helped me process my career decisions a bit better. I still may not know exactly what I will be doing come commencement day in June but, whatever I do, I will make it work for me! ▲

Maggie is 21 and has CF. She is a Director of USACFA and her contact information is on page 2.

HANLEY *continued from page 29*

detail will be given here. Suffice it to say that the information disseminated, learned, and of stories shared was wonderful and left much food for thought. Speaking of the food, it was great, maybe even more so due to the extraordinary company of members of the CF community at each table.

The banquet was a good example for me of the magical quality of the conference. At one point during the banquet, I noticed that the table next to ours had been winning nearly every

prize. I hopped over to that winning table and was invited to share a seat with one of the table’s occupants (no blue dot on her badge, phew! – so I was in the clear). While declaring I wanted some of that table’s luck to rub off on my table, I jokingly mimicked waving puka shells over their table. Returning soon after to my table – and this is true – my raffle ticket number was called. My husband and I had won a romantic Italian dinner basket!

Overall, the feeling at the confer-

ence was of amazement at the great information, good humor, positive comments and responses from others and, most of all, the understanding and support emanating throughout the conference. I really didn’t want to return to reality. So next year maybe I’ll bring some actual puka shells. Imagine the possibilities! Can’t wait... ▲

Jeanie is 47 and has CF. She is a new Director of USACFA. Her contact information is on page 2.

Transition of Care in Cystic Fibrosis

*Presented at the CFRI Conference
By K. Randall Young, Jr., M.D.,
Pulmonary and Critical Care
University of Alabama
Birmingham, Alabama
Reviewed by Rich DeNagel*

If you are following recent trends in CF Care, you're probably aware of the concept of transitions, which refers to a young adult with CF who transfers from a pediatric clinic and hospital into an adult-focused health-care environment. Dr. Randy Young, from the University of Alabama Birmingham (UAB), presented his ideas on the topic of Transitions.

Recognizing the need for transitions, the CF Foundation has mandated that all CF clinics establish an adult clinic with an adult pulmonary physician, or face losing accreditation. This reality creates a difficult challenge for CF centers. As is often the case, a change from the status quo reveals two opposing sides; one very positive and the other, not so much.

Dr. Young presented a model of transitions that is having great success at UAB. The first thing to consider is the level of expectations. Today, children with CF can expect to live into adulthood. If there is an expectation of living into adulthood, then transitioning will be a natural part of growing up and moving beyond pediatric

care. Currently, one of the challenges with transitions is that kids and parents hang onto their pediatrician and CF clinic out of fear of the unknown. The belief is that the pediatrician has kept the patient alive and so patients and families are scared to move on. So, the key is incorporating transitions from the beginning, from diagnosis, which will allow time to prepare for the change and create expectations of a positive outcome. No longer will we all associate this kind of change with preparing to die.

The next issue revolves around parents, a key factor in successful transitions. Communication with the parents is essential for a good transition and it's important that this begins at diagnosis. An integral part of the transition is including parents and family members, while encouraging patient assumption of responsibility. All programs should include a series of age-appropriate milestones that can be tracked and prepared for. Also, it's important that adolescent patients be seen alone for at least a portion of their doctor visit.

Finally, the entire pediatric CF team must be a part of, and supportive of, the transition process. One hospital nurse or RT or intern can sink the whole process.

Why do we need transitions? First, there are many issues and concerns facing teenagers and young adults that

cannot be appropriately addressed and dealt with in a pediatric setting. As CF patients grow older, they need to take responsibility for their treatment and care. Since we are living longer, we need to approach life like everyone else. On top of all that, there are issues that adult doctors have experience with and knowledge about that a pediatrician may not. It is important to talk about issues such as sex, alcohol use, and growing up, and an adult doctor will have a lot to offer. Plus, as we continue to age, we will begin to have medical issues that simply are associated with aging, rather than health issues that only involve CF.

The truth is - transitions are a good thing to have to deal with, since it means we are living longer. The reality that there are more adults with CF also means that we can expect more research focused on adults. But there are challenges involved in this new era of CF care, including getting qualified adult doctors, appropriate resources for adult CF clinics, and the natural resistance from pediatric doctors and clinics. However, if we keep focusing on expectations we are sure to move beyond these types of challenges. The good news is that the outlook is bright. ▲

Rich is 41 and has CF. He is a Director of USACFA and his contact information is on page 2.

POST *continued from page 21*

Camera info: D90 taken on November 6, 2008 on manual settings: 1/125 sec., F14, ISO 800, 18-200mm lens at 35mm.

Fall Whimsy

Another great place for viewing fall foliage is in Vermont. Bill and I came to a grinding halt when seeing these familiar characters in a roadside

nursery just down the road from our hotel. Every year the nursery delights in displaying whimsical scarecrows. A bus load of British tourists arrived a minute after us. A quick dash was necessary before the crowds invaded.

Camera info: D80 taken on October 6, 2008 on manual settings: 1/160 sec., F11, ISO 500, 18-200mm lens at 32mm.

Until next time, happy shooting; and just when you think you have been everywhere in your local surroundings, take another glance. You will be delightfully amazed. ▲

Pammie is 56 and has CF. She was a Director of USACFA for many years. She and Bill live in New Canaan, CT.

remember it.

14) What gets you through the tough days?

I would say the best thing to have for those days are visitors and friendly company. If I can't have that, I normally check out with distractions, like reading, surfing the web, watching TV, that sort of thing. Generally, the checking out method may make things worse if I don't stay on top of my medicines, so it requires a nice balance.

15) What do you hate most about CF?

I think what I hate most is that despite all the treatments and things I do to stay healthy, I still am not able to be as physically strong as other people. I can come close, but not quite equal.

16) What is your favorite movie? TV show? Why?

My favorite movie is "10 Things I Hate About You", a modernization of "Taming of the Shrew". My favorite TV show would probably be "The Simpsons". Both of these are absolutely hilarious, and I love to laugh.

17) Do you have kids? Want them?

I do not have kids, and I suppose it would be fun to raise one, but I'm a little young right now. I definitely don't want to have one while I'm in college.

18) What do you look forward to?

I look forward to a cure, or any new

drug that makes CF easier to manage.

19) Do you think having CF is a good thing or a bad thing?

Definitely not a good thing. Certainly it gives me a much different perspective but, hopefully, I'd be able to gain it without having such a disease.

20) Tell us about your friends?

Most of my friends are not the decision-making types, but that doesn't stop us from having fun and hanging out. Mostly we'll mess around on computers, play computer games, or play board games.

21) What is your favorite color?

Dark blue.

22) Do you spend time with other people who have CF? If so, what do you do, and how important is this to you?

I don't really spend too much time with other people who have CF. Mostly this is because I've heard too many cross-infection horror stories.

23) Do you spend time educating yourself about CF? How important is this to you? What effect does this have on your treatments? Rapport with your doctors? Self-image?

I don't spend too much time educating myself. I suppose that is because I already know the basics, and what would interest me most are new drugs

coming out that are targeted for those with CF. Mostly it is my doctor who would suggest a new drug, for example hypertonic saline.

So, that's a quick glimpse inside the world of brothers Brendan and Devin. They are a tremendous support to each other, and in speaking with them, it was clear to me that they are very close and important to each other. As I mentioned earlier, it's easy to forget the effect CF has on the people around us. From the anxiety of hospital visits and bouts with sickness to the constant wondering about how we're feeling and if we're taking care of ourselves, CF takes a toll on everyone in our lives. As I spoke with Brendan, I had this huge awareness that he was very in-tune with what was happening with Devin and understood the meaning and significance of what they are experiencing. And his responses were so heartfelt and honest. With that lesson from these two, I am going to work on remembering all those around me who show concern and help to take care of me, in ways both big and small.

If you want to be interviewed (and I need some new people to interview) drop me a line at my email address. ▲

Rich is 41 and has CF. He is a Director of USACFA and his contact information is on page 2.

INTERNET continued from page 17

ing is for free-floating or single bacteria, whereas cystic fibrosis lung infections and other chronic infections are caused by bacteria in a biofilm state which are much more difficult to kill than free-floating bacteria. The company also recognized that doctors regularly use combinations of antibiotics to treat CF lung infections, acknowledging the difficulty in killing biofilm bacteria. The result was bioFILM PA™, designed to assist doctors in selecting antibiotics for the treatment of chronic infections caused by the bacteria, *Pseudomonas aerugi-*

nosa. bioFILM PA™ is the world's first biofilm susceptibility test allowing doctors to make scientific based selection of antibiotics for biofilm infections like cystic fibrosis lung disease. In addition, bioFILM PA™ provides guidance on the selection of combination antibiotics, recognizing that doctors routinely use combinations since they know that single antibiotics rarely work on difficult to treat biofilm infections.

<http://blog.innovotech.ca/?p=42>

Hepatocellular carcinoma complicat-

ing cystic fibrosis related liver disease. D.H.O' Donnell, R. Ryan, B. Hayes, D. Fennell and R.G. Gibney. *Journal of Cystic Fibrosis*. Article in Press

Early diagnosis and treatment of the respiratory and gastrointestinal complications of cystic fibrosis (CF) have led to improved survival with many patients living beyond the fourth decade. Along with this increased life expectancy is the risk of further disease associated with the chronic manifestations of their condition. The researchers report a patient

Continued on page 34

with documented CF related liver disease, for which he was under routine surveillance, that presented with histologically proven hepatocellular carcinoma (HCC). It is important that physicians are aware of this association as increased vigilance may lead to earlier diagnosis and, perhaps, a better outcome.
<http://tinyurl.com/mot2ru>

Gene Linked to Liver Disease in Cystic Fibrosis

A variant of a particular gene in people with cystic fibrosis greatly increases their chances of developing severe liver disease, new research shows. About 3 percent to 5 percent of the 30,000 people in the United States with the condition will also develop a serious form of liver disease, including cirrhosis and portal hypertension, or high blood pressure caused by obstruction in the liver. Researchers from University of North Carolina at Chapel Hill analyzed nine variants in five genes previously implicated in cystic fibrosis liver disease. The researchers found that people who had the "SERPINA1 Z allele," or gene variation, had a five times greater chance of developing liver disease. The other variants did not increase the risk of liver disease. About 2.2 percent of people with cystic fibrosis carry the SERPINA1 Z allele, according to the study published in the Sept. 9 issue of the *Journal of the American Medical Association*. Screening for the gene variation could help identify those at risk of developing liver disease.

(SOURCE: University of North Carolina at Chapel Hill, news release, Sept. 8, 2009)
<http://tinyurl.com/ofgez>

Cancer risk among patients with cystic fibrosis and their first-degree relatives. Marie Johannesson, Johan Askling, Scott M. Montgomery, Anders Ekblom, Shahram Bahmanyar. *International Journal of Cancer*. Published Online: 23 Jun 2009

Patients with cystic fibrosis (CF) are

at increased risk of some cancers. Little is known about the cancer risks among carriers heterozygous for the CF mutation and it is hypothesized this may be associated with reduced cancer risk. Using Swedish general population-based registers, 884 patients with CF were identified from 1968 to 2003 and 3,033 of their first-degree relatives. Patients, followed for an average of 21 years, were at a higher overall risk of cancer. Statistically significant increased risks for kidney, thyroid, endocrine, lymphoma and non-melanoma skin cancer were found. There was no modification of cancer risk among parents and siblings, with an average of 21 years of follow-up. This study did not identify a heterozygote advantage for CF gene mutations in relation to cancer risk.
<http://tinyurl.com/l2sl3o>

Treatment Of Cystic Fibrosis: Encouraging New Results For Miglustat

Miglustat is a drug currently under phase 2 clinical trials in patients suffering from cystic fibrosis. In new work to be published on 1 August 2009 in the *American Journal of Respiratory Cell and Molecular Biology*, the researchers show that daily, long-term treatment of human cystic fibrosis cells with low doses of miglustat corrects the main pathological abnormalities. In the study, the researchers show that daily treatment of human respiratory cells that are homozygous for the F508del mutation with low concentrations of miglustat leads to progressive, sustained and reversible correction of the diseased phenotype. The researchers cultured diseased human respiratory cells in the presence of miglustat for two months. The correction observed in the cells takes place after 3-4 days, and then stabilizes. When the treatment is stopped, the cells revert to the diseased phenotype. The low doses used (3 micromolars) mean that they can be administered to patients and that their presence in the bloodstream causes no problems. This study is the first that shows that a cystic fibrosis cell can acquire a sustained non-diseased phenotype when treated daily

with a pharmacological agent.
<http://www.sciencedaily.com/releases/2009/07/090721090134.htm>

TREATMENTS

In vitro antimicrobial effects of aztreonam, colistin, and the 3-drug combination of aztreonam, ceftazidime and amikacin on metallo-beta-lactamase-producing *Pseudomonas aeruginosa*. Shigeharu Oie, Yumi Fukui, Masaya Yamamoto, Yuki Masuda and Akira Kamiya. *BMC Infectious Diseases* 2009, 9:123

Evaluation of in vitro antimicrobial effects on metallo-beta-lactamase-producing *P. aeruginosa* revealed relatively good effects of the 3-drug combination of aztreonam, ceftazidime and amikacin and marked effects of colistin.
<http://www.biomedcentral.com/1471-2334/9/123>

Intravenous monthly pulse methylprednisolone treatment for ABPA in patients with cystic fibrosis. Malena Cohen-Cymerknoh, Hannah Blau, David Shoseyov, Meir Mei-Zahav, Ori Efrati, Shoshana Armoni and Eitan Kerem. *Journal of Cystic Fibrosis*. Article in Press

Allergic bronchopulmonary aspergillosis (ABPA) in patients with CF is associated with frequent exacerbations and deterioration of lung function. Oral corticosteroids are standard therapy for ABPA and are associated with severe side effects. Monthly pulses of high-dose intravenous methylprednisolone (HDIVPM) are an effective therapy for autoimmune diseases with fewer side effects compared to oral prednisone, implicating its use for patients with CF who suffer from ABPA.
<http://tinyurl.com/nn9mct>

Recombinant activated factor VII for massive hemoptysis in patients with cystic fibrosis. Lau EM, Yozghatlian V, Kosky C, Moriarty C, Dentice R, Waugh R, Torzillo PJ, Bye PT. *Chest*. 2009 Jul;136(1):277-81.

Massive hemoptysis is a common
CF Roundtable ■ Autumn 2009

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- Send your questions of a general nature regarding legal issues that relate to CF to our legal advisor: **Beth Sufian, Esq., 811 Rusk Street, Suite 712, Houston, TX 77002-2807.**

INTERNET continued from page 34

complication in patients with cystic fibrosis (CF) and is associated with significant morbidity and mortality. Conventional treatment with antibiotic therapy and early bronchial artery embolization (BAE) is usually successful in achieving hemostasis in the majority of patients. Recombinant activated factor VII (rFVIIa), originally developed for use in

patients with hemophilia, has emerged as a general hemostatic agent that is potentially useful in the management of many life-threatening bleeding conditions. In this article, four patients with CF lung disease and massive hemoptysis were treated successfully with rFVIIa. In patients with CF who present with massive hemoptysis, the use of rFVIIa can be considered in

patients with refractory hemoptysis despite conventional therapy or as a temporizing therapy when BAE is not immediately available.

<http://tinyurl.com/l2jy60> ▲

Laura, 61, has CF. She is a Director of USACFA and is the President. Her contact information is on page 2.

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United Network For Organ Sharing (UNOS). Phone: **1-800-24-DONOR**. Call for information on transplant centers, access for all patients needing organ transplants and general transplant information.

Transplant Recipients International Organization, Inc. (TRIO): An independent, non-profit, international organization committed to improving the quality of life of transplant recipients and their families and the families of organ and tissue donors. For information write: **TRIO, 1000 16th St., Ste. 602, Washington DC 20036-5705. Or call: 1-800-TRIO-386.**

American Organ Transplant Association (AOTA): Helps defray out-of-pocket travel expenses for transplant recipients. Helps to set up trust funds. For more information write: **American Organ Transplant Assn., 3335 Cartwright Rd., Missouri City, TX 77459-2548. Or call (281) 261-2682. e-mail: infoAOTA@a-o-t-a.org.**

ADA: To learn how the Americans with Disabilities Act (ADA) applies to you, contact the Disability Rights Education and Defense Fund (DREDF) at **1-800-466-4232** between the hours of 9 a.m. and 3 p.m. Pacific Time. The DREDF has received a federal grant to inform individuals, who may be subject to discrimination, of their rights and remedies. To report a violation of the ADA, call the Equal Employment Opportunity Commission (EEOC) at **1-800-669-3362**.