CF Roundtable®
A NEWSLETTER FOR ADULTS WHO HAVE CYSTIC FIBROSIS SUMMER 2019

Don't Pick Up New Germs When Visiting An ENT

By Amy Sylvis


AMY SYLVIS

I've seen several ENTs across the U.S. for care and all, except for one, didn't gown and glove when treating me in an outpatient setting. I'm also pretty sure that the reusable blood pressure cuff, the chair, and the other surfaces in the exam room weren't wiped down between patients – all of this was despite these ENTs' affiliation with a CF center.

In the ENT setting, doctors are in pretty close contact with me, so why wouldn't healthcare providers follow the same contact precautions as CF centers? Cross contamination isn't isolated to just CF clinic visits – CF patients can easily transmit bacteria to each other in other locations like x-ray, blood lab, waiting rooms and, of course, other specialties (endo, GI, cardiology, ObGyn, allergy, ENT, PCP, etc.) where CFers attend.

I don't know about you, but I'm 38, and I work too darn hard on my health to catch bacteria from another patient due to lax infection prevention and control policies at my medical center.

Thankfully, the Cystic Fibrosis Foundation ("CFF") has clearly laid out guidelines for both CF centers and other non-exclusive CF areas of hospitals and medical centers in the outpatient arena. The guidelines[1] state that providers should "implement Contact Precautions (i.e., wear a gown and gloves) when caring for all people with CF, regardless of respiratory tract culture results, in both ambulatory and inpatient settings" and to "partner with IP&C (Infection Prevention & Control) teams to implement the recommendations in this guideline, *especially those that are likely to be followed in areas of the facility that are not dedicated only to people with CF* (emphasis added)." The full guidelines and recommendations can be found at: https://bit.ly/327I7ES

Sadly, my efforts to partner with an ENT to implement these guidelines haven't been easy. I've experienced ignorance, apathy, and hostility

[1]Saiman, MD, MPH, Lisa, et al. "Infection Prevention and Control Guideline for Cystic Fibrosis: 2013 Update." Infection Control and Hospital Epidemiology Vol. 35, No. S1, Cystic Fibrosis Foundation Guideline (August 2014), pp. S1-S67.

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INSIDE THIS ISSUE

CF ROUNDTABLE
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EDITOR'S NOTES

We are happy to tell you of two new directors of USACFA. They are **Mark Anthony Tremblay** and **Tré LaRosa**. You may read more about them on pages 26 and 27.

We also have some sad news of a former director, **Richard "Rich" DeNagel**. Rich, who had been both a director and a columnist, died in May. Please see his obituary on page 39.

Once again we have an issue that is full of good articles by a variety of authors. I hope that you have read the front cover where **Amy Sylvis** clues us in on the cross-infection protection guidelines from the CFF. This leads us right into the Focus topic of this issue, which is ENT Problems and Sinus Disease. **Jeanie Hanley** writes of vestibular problems. **Andrea Eisenman** tells of her six sinus surgeries. **Katie Lockwood** discusses her lack of a sense of smell. **Sydna Marshall** and **Brittany Wager** tell of their differing results from obliteration of the frontal sinuses. I bring up my sinus pain and surgery in "Speeding Past 50."

In "Ask The Attorney," **Beth Sufian** answers questions from readers about household deductions for SSI and overpayments of Social Security. **Isabel Stenzel Byrnes** talks about being *special* in "Spirit Medicine."

Be sure to check out the review by **Rob De La Noval** of the book — *Salt in My Soul: An Unfinished Life* — by **Mallory Smith**. Also, take a look at "Information From The Internet" that is so ably compiled by **Laura Tillman**. We find **Rebecca Mueller** "In The Spotlight."

Three people have contributed to "Voices From The Roundtable." They are **Alexis Schuller**, who writes of the monkey wrenches that CF can throw into our plans for our lives and how to work with that. **Mark Anthony Tremblay** writes of being sober for 32 years and working to help others. **Emily Trout** tells of her journey toward transplant.

I am using the final lines of this column to let you know that I no longer will be one of the editors of *CF Roundtable*. Although I have enjoyed working on this publication for 29 years, it is time for me to get out of the way and let younger, more qualified people do the work. I will still do my column, but I won't be involved in the everyday work of production of the newsletter.

I feel sure that there will be a space in my life that had been filled by doing things for USACFA that now will give me time for other things. I already have started on my backed-up sewing projects. Who knows, I might even get some of that huge pile of projects finished.

I know that Sydna and Andrea will continue to produce an outstanding newsletter. Their hard work is what makes it possible for all of us to have *CF Roundtable* to enjoy.

Stay healthy and happy,
Kathy

Publication of *CF Roundtable* is made possible by donations from our readers and grants from Sustaining Partners - AbbVie, Gilead Sciences, Two Hawks Foundation in Memory of Dr. Lisa Marino and Vertex Pharmaceuticals; Pearl Sustaining Partners - Boomer Esiason Foundation, Cystic Fibrosis Foundation; Diamond Sustaining Partners - Marina Day, Trustee of the McComb Foundation, Nancy Wech (in memory of daughter, Lauren Melissa Kelly & in honor of son, Scott Kelly).

Information From The Internet...

Compiled by Laura Tillman

Press Releases

How Will We Be Treating Cystic Fibrosis 10 Years From Now?

In 2015, a clinical trial was completed that showed that the function of the lungs of those patients receiving gene therapy did not decline as much as it did for patients receiving a placebo. It was the first time a gene therapy has shown clinical benefit in people with cystic fibrosis (CF). However, the improvement was moderate, so the researchers decided to modify the gene therapy to increase its efficacy. While the original version made use of tiny fat droplets to carry the DNA, the new one makes use of a hybrid virus. The researchers have modified a monkey lentivirus – a kind of virus that can insert genetic material in the host cell – to carry the external "targeting" proteins of a virus that naturally infects the lung. The new therapy has been tested in mice, where it has proved to be more effective than the previous version.

LAURA TILLMAN

The major challenge that led most gene therapies for CF to fail in the past is delivering the therapy to the lungs. To address it, researchers are now focusing on manufacturing the virus in large enough amounts to start clinical trials. If the therapy proves able to modify enough lung cells, the partners expect the therapy would need to be given only every 3-5 years, or even just once. It would also remove the need for any other medications for lung function.

Ensifentrine acts as both a bronchodilator and an anti-inflammatory agent in a single molecule. It blocks the activity of two enzymes involved in inflammation and is aimed at all CF patients independent from the mutation they carry. In addition, it has been shown to activate rare mutations of the CFTR protein, reducing the amount and thickness of the mucus. The treatment would not be a cure, but it has

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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 any about which you might like to write. In addition, humorous stories, articles on basic life experiences, short stories, artwork, cartoons, and poetry are welcome. We require that all submissions be original and unpublished. With your submission, please include a recent photo of yourself as well as your name, address, and telephone number. Photos will be returned. E-mail all submissions to: cfroundtable@usacfa.org.

Summer (current) 2019: ENT Problems And Sinus Disease.

Autumn (November) 2019: CFTR Modulators. (Submissions due September 15, 2019.) Have you tried any of the CFTR modulators? Are you currently using any of them? What results have you had? Do you have any suggestions for others? Please tell us of your experiences.

Winter (February) 2020: Insurance Issues. (Submissions due December 15, 2019.) Have you had any troubles with getting insurance or getting coverage that you need? Did you get help from anyone with dealing with insurance problems? Are you satisfied with your coverage?

Spring (May) 2020: Weight Issues. (Submissions due March 15, 2020.)

ASK THE ATTORNEY

Questions From Our Readers, Answers From the Attorney

By Beth Sufian, J.D.

CF Roundtable readers asked questions related to the Social Security Household Deduction for those receiving SSI benefits. Readers also asked questions about Social Security overpayments.

No reference made in this column is to a specific situation. Nothing in this column is meant to be legal advice about a specific situation but is meant to be only information.

The CF Legal Information Hotline can answer questions related to Social Security benefits, Medicare, Medicaid, health insurance, and rights in employment and education. If you have questions, please e-mail CFLegal@sufianpassamano.com to schedule a time to speak with an attorney.

Why does Social Security deduct one-third of my SSI check resulting in me receiving only $540 for my monthly SSI check? I know other people with CF who receive an SSI check of $771 a month.

In order for an adult to receive the full SSI monthly benefit amount, the SSI recipient has to pay his share of rent and utilities and have enough left to pay for monthly food costs with his SSI check.

If someone else pays the rent, utilities and/or food expenses for a person who receives SSI benefits, then Social Security deducts one-third from the monthly SSI check. If rent, utilities, or food are paid for by another person, then Social Security will impose something called the Household Deduction.

In June 2019, the federal maximum monthly SSI check is $771. New York and California supplement the federal SSI amount with state funds, which increases the amount of the benefit SSI recipients receive in NY and CA.

If a person has a roommate and the total rent is $1,100 then each roommate should pay $550 for monthly rent. When $550 is paid for rent, $50 for utilities and $150 for food, then the person has $750 for household expenses and still has $21 left. This would mean the person can pay her household expenses and there would be no Household Deduction imposed by Social Security.

However, if the rent was $2,000 a month and there were two roommates, the share of rent would be $1,000 for one roommate. Since the SSI monthly check is only $771 in the majority of states, then the person cannot pay his share of household expenses with his SSI. Social Security will impose a Household Deduction and the person will have one-third of his SSI check reduced each month. If the SSI check would have been $771 a month, the SSI check will be reduced by $240 a month.

BETH SUFIAN

I received a Notice of Overpayment from the Social Security Administration what can I do?

An overpayment is what happens when you receive more money from Social Security than you should have received in a certain month. There are six common reasons for an overpayment of benefits:

1. Social Security learns a person has earned money from work activity that was not counted when Social Security determined the monthly Social Security benefit amount. This usually happens when someone starts part-time work after a period of not working at all or increases the number of hours worked part time.

2. A person's living situation changes and she is not able to pay her share of household expenses. If this happens to a person who receives SSI benefits, the person's benefit should be reduced by one-third under the Household Deduction Rule (see the first question above for an explanation of the Household Deduction Rule).

3. A person who receives SSI gets married and the new spouse's income or assets makes the person with CF ineligible for SSI benefits.

4. A person on SSI benefits goes over the resource limit. For an individual, the SSI resource limit is $2,000; and for a family of more than one person, the resource limit is $3,000.

5. Social Security determines a person no longer meets the medical criteria for Social Security benefit eligibility, but the Social Security benefit checks are issued because the computer system has

not yet registered the change in eligibility status.

6. Social Security incorrectly figures a person's benefit amount because of incorrect or incomplete information provided by the person with CF.

Once Social Security determines there is an overpayment, a Notice of Overpayment is sent to the Social Security recipient. The notice requests payment of the amount listed. Once a Notice of Overpayment has been received, the person should contact Social Security as soon as possible. If the person wishes to appeal, he must appeal within 10 days of receipt of the overpayment notice to avoid having the monthly benefit check being stopped pending payment of the amount listed in the notice.

A Social Security recipient can do one of the following:

1. A person can appeal the overpayment and provide proof that Social Security is wrong and the person does not have an overpayment of benefits. If a person appeals more than 10 days after the receipt of the Notice of Overpayment, Social Security can stop benefits entirely until the appeal is decided. In some areas of the country, it can take one to two years to have an appeal decided by Social Security.

2. A person can ask for a waiver of the overpayment at any time. The person must show that it was not her fault that she was overpaid by Social Security, and that she cannot pay back the overpayment because she needs the money to meet ordinary living expenses. Social Security can require the person to submit bills to show that her monthly expenses use up all of her income, and that it would be a hardship if she had to repay the overpayment amount.

3. If the request for waiver is denied, a person can ask for a reconsideration. If the reconsideration is denied, then an appeal can be filed requesting a hearing before an Administrative Law judge.

4. If the waiver is ultimately denied by a judge, or an appeal is denied by a judge, then SSA can withhold the full amount of a Social Security benefit check until the overpayment is paid back in full.

5. If a person no longer receives SSI or SSDI benefits, then Social Security may withhold the overpayment amount from a Federal Income tax refund or from any Social Security benefits a person may receive in the future.

6. If, upon receipt of a notice of overpayment, a person determines he owes Social Security money, he can request a payment plan or request a certain amount of money be withheld from his monthly benefits. If a request for a payment plan is not made early on in the process, SSA may determine they will not agree to a payment plan. Recently it seems it is difficult to get Social Security to agree to a payment plan after a denial of a waiver or a denial of an appeal. Social Security does not have to agree to a payment plan but seems more likely to do so if the offer to make payments is made early on in the process. ▲

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*Beth is 53 and has CF. She is an attorney who specializes in disability law and is a Director and current Vice President of USACFA. Her contact information is on page 2. You may contact her with your legal questions about CF-related issues at CFLegal@*sufianpassamano.com

What is the Boomer Esiason Foundation?

In 1993, NFL Quarterback, Boomer Esiason, learned that his son, Gunnar, was diagnosed with the incurable genetic disease cystic fibrosis (CF). Never ones to back down from a fight, he and his wife, Cheryl, founded BEF and decided then and there to fight for a cure and for the cystic fibrosis community.

Cystic Fibrosis is an inherited chronic disease that affects the lungs, digestive system, and reproductive system of about 30,000 Americans by causing a thick build-up of mucus that leads to blockage, inflammation, and infection.

What does BEF do?

In addition to assisting the CF community with the following programs, we also support CF clinics and research centers:

- **Educational Scholarships**
- **Lung Transplant Grant Program**
- **Team Boomer**
- **Jerry Cahill's Cystic Fibrosis Podcasts & Wind Sprints**
- **Breathe In Podcast**
- **CF Patient Disaster Relief Program**
- **CF Step by Step Video Series**
- **Gunnar Esiason Blog**
- **Tru Heroes Nursing Program**
- **You Cannot Fail Hospital Bags**
- **CF Education Days & CF Speaking Engagements**

www.esiason.org

SPIRIT MEDICINE

The Spirit of Specialness

By Isabel Stenzel Byrnes

I am writing this article in New York City, and just saw the musical *Oklahoma*. The show won the Best Musical Revival and made history when Ali Stroker became the first person in a wheelchair to win a Tony Award. I wondered if she won a Tony because she was in a wheelchair, or because she was an exceptional performer. In my opinion, it was the latter — she was amazing! Yet her disability has made her special, along with her talents. I decided to use this Spirit Medicine article to explore what it means to be special when you have cystic fibrosis. I'd like to ponder how being special because of CF impacts our spirits.

Each time I travel, and approach the gate to board the plane, I hear the familiar announcement inviting people who need special accommodations to board early. I remember a friend with CF telling me she always boards early. Should I? I am wearing a mask and wonder if this marks me as medically fragile in some way. If I boarded early, I could avoid the swarm of people gathering around me. I could put my bag full of medical equipment into the overhead space without worrying whether it will fit. Yet I don't have a mobility impairment. I don't need help from others. I don't like special treatment. So, I wait in line, with group 5, and board like cattle alongside everyone else who got a bargain ticket.

I remember a card that my best friend gave me for my sweet 16th birthday. It said "[b]eing special means being different but not too different." It summed up my ambivalent relationship with cystic fibrosis. I wanted to be treated normally and live a normal life. Yet I wanted my CF to be affirmed, because it had already shaped my life in so many tough ways. I'm grateful for how this disease has made me special — giving me unique life experiences and perspectives. Yet I also reject CF as a source of specialness; I've wanted to create my own specialness through intrinsic and developed skills and talents, passions, and my own volition.

I believe being special nourishes the spirit. Everyone wants to be recognized and valued for who they are in their own unique ways. Most people want to be special for something that gives them self-esteem and identity. Some people are special because of things they are born with — they are exceptionally smart or talented at music or math. Some people might develop a talent and become more special — like an Olympic athlete or opera singer. With hard work and opportunity, they set themselves apart from all of us and create a name for themselves.

CF makes us special, but it's not a form of specialness that we choose. It sets us aside from others and makes us different. But I believe what we choose to *do* with our lives with CF is what creates our specialness. I just received a copy of Andy Lipman's book, *The CF Warrior Project*. In it are 65 stories of people with CF from around the world who are doing extraordinary things. There is a circus acrobat, a man who climbed some of the highest peaks in the world, there are actors, there are professional athletes, marathoners, firefighters and police officers, musicians, and professionals. These people are exceptional human beings making the most of every day — and they happen to have CF.

> *"CF makes us special, but it's not a form of specialness that we choose."*

ISABEL STENZEL BYRNES

I remember at CF camp back in the 1980s, a 25-year-old woman with CF, my counselor, was getting a Ph.D. I was about 12 and said, "Wow, you must be very smart." She responded, "No, it's just compensation for my CF." Huh? I didn't get what she meant, but now I do. She was achieving something important for her, perhaps because her disease was taking so much away. The word *compensation* still sticks in my mind. If we have to deal with the burden of CF, do we deserve some source of pride, goodness, or relief in comp-

ensation? Do we deserve to board the plane early?

We can choose how to incorporate this specialness of CF into our lives. I chose to write a book and be in a movie about living with CF, for example, as well as compete in the Transplant Games. So many sources of my identity and self-esteem are CF derived. I wonder what my life would be like without CF. What would make me special then?

But I also chose to become a social worker, thanks to CF's lessons. The Americans with Disabilities Act has made it much easier for people with serious illness and disabilities to receive accommodation to help them work. I strongly believe we deserve a chance to earn a living. Disabled persons deserve some compensatory assistance to help them reach their fullest potential. But the danger of "specialness" comes when a person with an illness or disability expects to be supported and aided in excessive ways, and feels entitled to attention, pity, and over-the-top accommodations. This can be draining and maladaptive in the work environment. I don't ever want to use my specialness in this way. I don't want to take advantage of having CF.

Like many of you, CF impacts almost every part of my life. Thankfully, my lung capacity is good, but I have a complex medical regimen I must follow to stay healthy. I am blessed to live a very normal life and no one could tell from the outside that I have health issues. I don't take anything for granted and I count my blessings every day. Yet, in my work life, I am just like everyone else — stuck in traffic, meetings, advocating for my department, and getting frustrated over bureaucracy. It's a humbling experience to fit into the ordinary working world, which my CF prevented me from doing for so many years. And at work, I'm not special because of my CF. I am special because I work hard and do a good job.

Being special requires a delicate dance between sharing our specialness due to CF and keeping it private. If we share it, we are vulnerable to rejection or judgment, especially in a job setting. We are also open to compassion, kindness and being told we are an inspiration. We are subjected to being told we are amazing because we just are living our lives and also happen to have CF. If we keep our CF private, no one will know what we have to go through and the unique wisdom we have gained because of this disease. But they will probably see a specialness within us that has nothing to do with illness. That's also a good thing.

If we feel special, and we feel good about ourselves, our spirits are uplifted. We are using the gifts and resources given to us from God or the Universe to our best ability. I believe God creates each and every one of us to be special in our own right. He created us in our own unique form, to be exactly as we are. We do not have to be overachievers or award winners. I live in the very competitive culture of Silicon Valley, where young professionals are trying so hard to prove themselves, to overwork and make more money than their neighbors. They are trying so hard to be special. And I notice this effort comes with a cost. I believe God loves us unconditionally, simply for being good, faithful people, caring, and helpful to others. When we have CF, we can do just that. We can also live the best life possible with our disease, whether we are working or on disability. It is hard to feel special for just *being*, rather than *doing*, in this culture. This is a cliché, but we are human beings, not human doings, after all! But really accepting and believing this can foster a healthy spirit.

So, what makes you feel special? How do you choose to let CF into your specialness? Like Ali Stroker, I hope you are recognized by people around you for the talents and skills you have cultivated and worked hard for...and you happen to also have CF. ▲

Isa is 47 and has CF. She lives in San Mateo, California, with her husband of 20-plus years, Andrew. She can be reached at isabear27@hotmail.com.

Benefactors

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SPEEDING PAST 50

It Was All In My Head

By Kathy Russell

It has been so hot here for the past few days that I am really happy that I no longer have serious sinus problems. When my sinuses were bad, hot days only made the pressure and pain worse. I can remember days where it felt as if my pain would be eased if I just pulled all of my teeth and squeezed my head really hard. Of course, an ice bag that covered my entire head might have felt good, too. I was unable to bend over and it didn't feel good to lie down. I was miserable. I suffered with sinus pain for most of my first 50 years of life.

In 1979, I had the first of my sinus surgeries. At that time, surgeons were still going into the sinuses by making incisions inside one's mouth, above the teeth. From the sinus X-rays that had been taken, it was easy to see that I was born with only one frontal sinus and that my troubles were from my ethmoid, sphenoid, and maxillary sinuses. These are the sinuses on each side of one's nose and cheeks as well as above the ears. In other words, I had pain in all of the front half of my skull.

The surgeon had to cut holes through my skull into my sinuses below both cheekbones. He got out thick, infected "gunk" and balls of mold and fungus. It's no wonder that I had kept sensing a smell that seemed rotten.

After the surgery, I had a couple of doozy black eyes! My face was swollen and black, yellow, green, and purple. But the relief from pain was amazing! The nurses could not believe that I refused all pain meds. I hurt so much less after surgery than I had before, that I simply felt there was no pain. It took a few weeks for my face to look like me, again, but I was so happy to not have sinus pain.

Sadly, it didn't last long. I had to have surgery again two years later. At least I didn't have to go through quite so much discomfort with the second surgery. They didn't have to cut holes in my head that time. I didn't turn rainbow colors, but I did experience some pain right after the surgery. Again, the pain in my sinuses was eased. I thought that maybe I was "home free." Alas, that was not to be.

By 1995, I was having serious sinus issues, once again. The pain was back with a vengeance. I was smelling something that was quite awful all the time. My doctor tried to lavage my sinuses through the holes into my maxillary sinuses. He was unable to gain access because the holes had grown shut. That meant that I had to have another surgery.

I had laser surgery that time. That was so much easier. Thank goodness for advances in medical practice. My ENT doc was surprised at how much of the fungus and mold had grown up in my sinuses, once again. He hoped that we might get a "good fix" with this surgery.

> *"It took a few weeks for my face to look like me, again, but I was so happy to not have sinus pain."*

KATHY RUSSELL

During the procedure he trimmed my turbinates, which caused me to lose all sense of taste and smell. I always had enjoyed a very fine sense of smell and could identify aromas quite easily. I could reproduce a recipe from just tasting a dish. All of a sudden, I couldn't even taste food. I perceived only salt, sweet, sour, and bitter. Subtle nuances of seasoning and flavor were completely lost on me. I really missed those senses. However, I still could smell some bad smells. Wouldn't you know that of all the aromas there are in this world it would be the bad ones that I could perceive? But I wasn't in pain.

When the doc checked me out as I was healing after surgery, we talked about doing something to prevent recurrences of sinus infection and pain. He decided to leave in the two irrigation tubes that he had placed in my sinuses and use them for tobramycin irrigations. He continued the irrigations for a while before removing the tubes. Then I continued the irrigations

manually for another week or two.

After I finished with tobramycin, I began to irrigate my sinuses with sterile normal saline and a mild glacial acetic acid solution. We had to work at it to find the correct amounts to use of each. I finally found the amounts that worked and I continued using those doses until just a few months ago. That was about 24 years of using the same solutions and rarely missing a dose. It has worked extremely well for me. As a matter of fact, I have had only a couple of sinus infections in the past 24 years. I think that is great!

Now, my doc has me using a hypertonic saline solution for irrigation. He wanted to make some changes and I am willing to try something new. It seems to be working just fine, but I rather miss my old standby solutions. I was comfortable with them and was a bit leery of changing anything. I don't like how the hypertonic saline feels in my throat. It seems to be rather harsh. I imagine that I'll get used to it.

We'll see how I'm doing when I see my doctor after another month. He wants to make sure that I don't have polyps growing, since I haven't had any grow for the past 24 years. I sure don't want to have to deal with those things growing again. Wish me luck! ▲

Kathy is 75 and has CF. She and her husband, Paul, live in Gresham, OR. She is a past director of USACFA and is the Managing Editor of CF Roundtable. *Her contact information is on page 2.*

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potential to treat the symptoms and improve the quality of life of people with CF. The treatments they currently receive aim to repair the defects caused by specific mutations in CFTR proteins, but their effect can be limited and patients still see their lungs getting inflamed and infected.

Another pharmaceutical company is developing drugs targeting the mucus that causes the airways to become blocked. In particular, the company targets ion channel proteins that control fluid secretion and mucus clearance from the airways. Its two leading programs are in preclinical testing, and the company expects to start the first clinical trials for both in early 2020.

It is important to note that while many new approaches in development for CF bring hope to improve the lives of people with this condition, most of these efforts are limited to the effects that the disease has on the lungs. While lung complications are the most severe symptom of CF, medicine is starting to look more closely at the effects CF has on those other organs, such as the bowels, the pancreas and the liver. Researchers are working on the development of miniature organoids that replicate the structure of different tissues using cells from patients with CF. This technology could yield results that are much more accurate at predicting how the human body will behave under different conditions and how it will react to experimental drugs. It could also help patients with rare forms of CF be prescribed a personalized treatment.
https://tinyurl.com/y3o3ec5c

Window For Treatment Of CF Lung Infections Is 2 To 3 Years, Study Says

Treatment of Pseudomonas aeruginosa lung infections in cystic fibrosis (CF) patients could be more effective if done within a crucial two- to three-year period when the bacteria are still susceptible to antibiotics, according to a study. To better understand pathogen adaptation to the host, researchers used statistical methods that account for the body's effects on bacterial lineages and assessed their adaptive paths. Results revealed that P. aeruginosa rapidly adapted within two to three years after infection, a period in which they showed little antibiotic resistance. The bacteria then grew slower and reduced their susceptibility to ciprofloxacin, an antibiotic commonly used as first-line treatment in CF.

This variability in bacterial population and modes of adaptation contributes to pathogen persistence in the airways, according to the investigators. The findings also support the idea that bacterial adaptation is patient-specific, and is influenced by specific mutations and genetic background. Treatment resistance is independent of whether or not the bacterial strains are mucoid — a trait characterized by the production of a protective, slimy coating and associated with greater difficulty in fighting the pathogen and accelerated loss of lung function. In contrast, traits such as the ability to attach to surfaces evolve more consistently in persisting infections, and could be a better early marker of chronic infection.
https://tinyurl.com/y2u9cxjy

And
https://tinyurl.com/y2tfrm64

New Test Could Lead To Personalized Treatments For Cystic Fibrosis

In a new pilot study, researchers developed a novel, straightforward way to test multiple drugs on cells obtained from individual patients with cystic fibrosis (CF), raising the possibility of highly personalized drug treatment. The test combines high-speed video microscopy with a novel video analysis algorithm to measure the coordinated movement of cilia — hair-like structures

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toward what one patronizing doctor called my "special needs." I was also threatened to be kicked out of a practice. All for just trying to protect myself and my community from deadly, multi-drug-resistant germs.

But I didn't let that stop me. Here are a few workarounds I've learned along the way when trying to implement CFF IP&C Guidelines. It has taken a while but, so far, I've been successful.

> "I don't know about you, but I work too darn hard on my health to catch bacteria from another patient due to lax infection prevention and control policies at my medical center."

- I have discussed with my non-CF doctor/nurse/medical assistant why cross infection is an issue in cystic fibrosis and what acquiring a new, virulent, multi-drug-resistant bacteria could mean to my health. I make sure to explain not only the science behind my request, but also the personal side of infection control — what my life would be like if I acquired a new multi-drug-resistant, deadly bacterium (more time in the hospital, more missed work, more coughing blood, less time with friends and family, more breathing treatments).
- I have discussed what steps the CFF recommends based on extensive research to protect CF patients from cross infection in the 2013 Infection Prevention and Control Guidelines for CF. I've printed out and e-mailed the actual guidelines and pointed to the sections in the guidelines that I'm hoping he/she will partner with me on — namely, the section discussing the outpatient ambulatory setting (gown and gloves for not only doctors but also medical assistants, nurses, x-ray techs, phlebotomists, etc.).
- If the treating physician/nurse/MA/x-ray tech/phlebotomist is apathetic, ignorant, name calls. or threatens to kick me out of their practice, I haven't let that stop me. There are other options. I have reached out to the IP&C department of the healthcare center (hospital, medical group, etc.) — a simple Google search or talk with the oper-

TILLMAN continued from page 9

covering airway cells that remove mucus from the lungs and upper airways. The research team used their test to study the movement of cilia in cells derived from multiple patients with different CF mutations, comparing those samples to normal cells. They then measured the response of those cells to six different drug treatments, including ones not currently approved for CF. The team found that the patients responded differently to the drugs, suggesting that what works for one person might not work for another, even if each carries the same mutation. Going forward, the researchers hope that their test could be used to recommend more personalized treatments for this life-threatening disease.

https://tinyurl.com/y3mlgr2e

AND

https://tinyurl.com/y6xysgrr

AND

https://tinyurl.com/yykjjuc4

In CF, Early Antibiotic Treatment Better To Eliminate New MRSA Infection, Study Says

Using antibiotics to fully eliminate new methicillin-resistant Staphylococcus aureus (MRSA) airway infections appears more effective than waiting for the infection to pass naturally in patients with cystic fibrosis (CF). In addition, this approach may also provide benefits for the patient's respiratory function and nutritional status. The results of this study agree with previous experiences regarding the possibility of eradicating new-onset MRSA infection, and show favorable effects in CF patients' FEV_1 and body mass index (BMI) over a period of 6 months. These results, together with other data from the literature and the low risk of side effects of the treatment, suggest that this strategy could be more widely implemented in the treatment of CF patients.

https://tinyurl.com/yyfdlnq6

Amphotericin Holds Promise As Treatment For All CF Patients, Preliminary Study Shows

Amphotericin B, a widely used antifungal medication, may be a potential treatment to restore lung mucus properties, including its ability to fight infections. The medicine creates pores in the membrane of cells lining the airways, working as a substitute channel for CFTR, which is the protein missing or defective in people with CF. Researchers demonstrated that amphotericin restored the flow of bicarbonate ions out of lung cells, bringing the pH levels, viscosity, and antibacterial activity of the airway surface liquid back to normal. Researchers say the therapy may

ator of the main telephone number helped me find this department. Not only is the IP&C department really interested in patient safety because that's their job and they're human, these departments exist to protect the hospital/medical group from legal liability from improper attention to IP&C. Which means, they're often super receptive to hearing about my needs as a cystic fibrosis patient. Again, I make sure to explain not only the science behind my request, but also the personal side of infection control.

• I have also reached out to the CFF to ask for support in working with the medical system/hospital to implement the CFF's IP&C guidelines. They have contacts, relationships and, of course, accreditation power that has been useful to me in implementing these guidelines. It's unfortunate, but sometimes an organization has more credibility in the minds of physicians and hospital administrators than patients.

• I have not had success in this area, but I know others have had success in partnering with the medical center/hospital's CF center to leverage relationships to implement IP&C in non-CF exclusive areas. It's disappointing, but sometimes physicians speaking to other physicians can have a more powerful impact than patients advocating to physicians or hospital administration.

• I haven't been able to leverage this either, but I know others who have been able to get other CF people who attend the medical center/hospital to advocate for IP&C change with them. There is no question that a group of voices advocating for the same thing is infinitely more effective than a lone wolf voice. If you don't know anyone in your CF center, create a FB or Reddit group for your center and get to know each other so you can advocate for your needs.

At the end of the day, no one cares about your health more than you do. Advocacy work can be tiring, scary, and intimidating — but don't ever forget that you have rights as a patient and you are worth a safe environment, both in and outpatient, when receiving healthcare.

Please don't hesitate to reach out to me if you want to discuss my experiences or want to partner in implementing changes at your medical center/hospital. We are stronger together. ▲

Amy is 38 and has CF. She is a director of USACFA. Her contact information is on page 2.

hold promise as the first treatment suitable for all types of CF, regardless of the underlying CFTR mutation. Using cells that line the lungs — airway epithelial cells — the researchers found that amphotericin B can form channels at the surface of those cells, releasing the bicarbonate stuck inside them. The treatment also helped normalize pH levels and the thickness of the airway mucus. The ability of amphotericin to bypass the CFTR defect and create new channels means it may be able to treat CF patients who completely lack CFTR, for whom no treatment exists yet.

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New Method Catches Cystic Fibrosis Inflammatory Enzyme In The Act

A combination of two techniques offers a clinically feasible way to measure inflammatory response in chronic lung diseases. People with chronic lung diseases such as cystic fibrosis (CF) are caught in a vicious circle: infections cause inflammation and immune cells rush into airways, but in the process of killing infectious bacteria, some enzymes remain bound to the surface of the immune cells that secreted them, so they cause further tissue damage and inflammation.

A new method allows researchers to track this inflammatory enzyme activity in patient cells. The method, which relies on a combination of fluorescence-based approaches, could help monitor how patients respond to therapy. Researchers developed the technique to measure the activity of cathepsin G, one of several proteases secreted by immune cells known as neutrophils.

In the new study, the team tracked cathepsin G with a method for detecting peptide interactions. To make use of the technique, the researchers first used flow cytometry to isolate cathepsin G-secreting neutrophils from other cells in sputum samples from people with and without CF. They then carried out the FRET tests on the neutrophils and found that the cells of people with CF showed approximately three times as much cathepsin G activity than control cells.

https://tinyurl.com/y2ka7ekx

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FOCUS TOPIC

ENT PROBLEMS AND SINUS DISEASE

Buzzed

By Jeanie Hanley

I used to have perfect hearing and great balance, but then I got buzzed. After years of many hospitalizations and home IVs all with courses of tobramycin, I began losing my balance more frequently, and I developed incessant buzzing in my ears. And it wasn't due to alcohol or any illicit drug. I initially thought the imbalance was due to a recurrent tendon tear in my left ankle that had left my left shin weaker than my right. So, I started PT and the astute physical therapist figured out that the problem was not in my left shin, but was due to vestibular ototoxicity (literally meaning ear poisoning) causing tinnitus (pronounced either ti-NIGHT-tuhs or TIN-nuh-tuhs), imbalance, and a host of other effects.

Vestibular ototoxicity is due to damage of the inner ear or the 8th cranial nerve, also called the vestibulocochlear or auditory vestibular nerve or both. The nerve courses from your brain through your skull to your inner ear. It transmits information about hearing and balance received by the inner ear back to the brain. When the nerve or inner ear structures are damaged, symptoms like hearing loss, tinnitus, vertigo, dizziness, and imbalance commonly occur, all of which have been an issue for me.

For those of us with CF, vestibular disorders and resulting sensorineural hearing loss are a significant issue. It is known that 35% of those without CF who are over 40 have some symptoms of vestibular dysfunction. Most with CF are under 40, where the incidence of ototoxicity varies widely in CF studies and among CF centers. We do know that we are more vulnerable to vestibular damage and hearing problems than the general population due to our frequent exposures to aminoglycoside antibiotics. The older you are, and the more hospitalizations with ototoxic antibiotics you have experienced, the greater your risk of getting buzzed.

Aminoglycoside antibiotics, like tobramycin and gentamicin, can directly damage the inner ear and nerve. Other causes of vestibular damage are exposure to loud noises and TMD (TMJ disorder). The temporomandibular joint (TMJ) has associations with the auditory nerve and connections to the ear drum and inner ear. If you suffer from TMD, then it can aggravate the vestibular system even more. All of these have no doubt played a role in my ear issues, the greatest of which (apart from my ripe age of 57!) was frequent exposure to tobramycin, which kept my lungs in good shape for a very long time. Unfortunately, I can no longer take tobra due to the real risk of worsening vestibular damage.

JEANIE HANLEY WEARING NOISE-CANCELING HEADPHONES.

Although physical therapy was very helpful in strengthening my core and lessening the dizziness, the buzzing in my ear progressed from the right ear to the left, and then got louder, along with hearing loss. With my equilibrium off more often, I have to work harder to maintain my balance and truly be in the moment and put all my focus on walking. It's very difficult to maintain balance while making sudden active movements (I fell to the ground once while serving a volleyball on the sand). I feel like a pinball while walking in crowds or on narrow sidewalks. Airplane aisles are the worst — being narrow obstacle courses where maneuvering around passenger arms and legs is necessary. The bumpy rides and turbulence don't help, either. I have to hold on to seatbacks as I walk to the back of the plane. On sidewalks, I have to hold someone's (usually my husband's) arm to maintain the best balance. What helps me to walk in a straight line (more or less) when I'm by myself, and to not look like a drunken sailor, are looking up and ahead (even while talking to someone beside me) and tightening my abdominal core and inner thigh muscles.

Because it's often difficult to hear over the buzzing in my ears, I try to use different ways of saying "what?" when I can't hear someone's soft voice or I've heard it incorrectly. Instead, I'll say "How's that?" or "Tell me again" to try to hide the fact that I really mean "what?" It doesn't really work, but I try. This issue is magnified if I'm in a noisy place. Hearing incorrectly is a frequent issue and I've had to chuckle to myself and with others (because you need a sense of humor and just have to go with it sometimes) by repeating what I think they said. "You're going to see a

sick mouse?" when they actually said, "we're going to see Mick-ey Mouse." Oops. Got buzzed.

One other problem with hearing loss is that it does separate you from people if you can't hear a conversation or if you hear it poorly or incorrectly. Asking people to repeat themselves is tedious and sometimes I don't even bother. This can lead to depression and cognitive decline. What helps is to pick locations — for example, in the case of restaurants: outside seating, or booths that are far away from cash registers or the busy kitchen. Choosing an area with soft music and low traffic is all the better. I seat myself so that my companion is speaking to my left side since my left ear hears best. If it's a larger number of people, I sit at the head of the table so that I don't have to turn my head as much (which can throw off equilibrium) and this allows me to read lips, too (or try to, anyway). Sitting in the front of an auditorium also helps. Even so, sometimes it just plain bums me out that I didn't fully hear the information, a conversation, story or, worse, I missed the punchline of a joke. And I love a good joke so that's quite a bummer and buzzkill when asking someone to repeat a punchline. Maybe next time someone asks you to repeat a punchline, you'll have a little mercy.

Other problems with ototoxicity are sound and visual sensitivity. When music or movies are loud, it's very discomforting, and I have to leave the room or put in earplugs. There is also visual sensitivity with vestibular disorders. It's easier to lose my balance at nighttime when walking; along the same vein, driving at night and along bumpy roads at any time of day will cause quite a headache from trying to focus so intensely. If I had a superpower, I could burn holes through the road with my intense focus at night.

Some treatments that have helped are tools that I learned in physical therapy — balance strengthening of the abdominal core and vestibular rehabilitation. The rehab consists of many exercises of eye tracking and other reflexes that trick the brain to learn to improve focus and maintain balance while you're in motion.

Other helpful tools are carrying small earplugs with me always just in case a place turns out to be very buzzy. To prevent further damage, I use noise-cancelling headphones while using loud devices like the Vest or during nebulizer treatments. I have a mouth guard to treat and prevent TMJ. Sometimes I use a walking stick, especially during hiking or when I plan to be in a crowd.

There are some tricks that I plan to try. For example, glacier sunglasses are helpful since they have side visors that minimize distraction from peripheral vision. A wide-screen computer can minimize scrolling through documents and reduce the visual sensitivity. Seeing two documents at the same time reduces head movement and fatigue. To stay on top of it and motivate me to keep trying different things, my hearing is tested annually with visits to the ENT and audiologist.

The good news for those of you who don't have ototoxicity is that Sound Pharma has a new therapy in development, SPI-1005, that prevents ototoxicity when used prior to receiving an aminoglycoside antibiotic. As of this writing, their STOP Ototoxicity clinical trial is in Phase 2. If you're fortunate, you will never have to deal with this. If, unfortunately, you do, I encourage you to try some therapies mentioned above and maybe you won't get buzzed. For more information, visit the Vestibular Disorders Association at vestibular.org. ▲

Jeanie is 57 years old and has CF. She is a physician and President of USACFA. She lives in suburban Los Angeles where she can stroll the wide streets and quiet neighborhoods with her husband John.

CF Survival Kit

Hospital stays become a routine way of life for people with CF. They can get lonely and depressing. The BOOMER ESIASON FOUNDATION and YOU CANNOT FAIL will send some inspiration, hope, and education to those in the hospital and BEYOND.

For your CF Survival Kit, email cmcewan@esiason.org

Frontal Obliteration Sinus Surgery: All Your Questions Answered

By Sydna Marshall and Brittany Wager

> "Instantly, I could smell things I hadn't smelled in years. My ability to taste changed. It was the greatest feeling. — BW"

In the following question-and-answer interview, two CF patients discuss their shared experiences undergoing frontal obliteration sinus surgery. Frontal obliteration sinus surgery is the removal of the frontal sinuses by way of the skull, followed by the insertion of fat tissue from elsewhere in the body to keep the sinus cavity from regrowing. It's roughly a seven-hour surgery, with an extensive recovery period, and is typically considered a last-resort surgery for individuals with severe sinus disease.

Brittany Wager (BW) and Sydna Marshall (SM) are both CF patients living in Austin, Texas. Both see the same CF doctor (Dr. Jason Fullmer) and the same ENT specialist (Dr. James Eskew).

1. Tell us a little about yourself. How many sinus surgeries have you had in total? When were you diagnosed with CF?

BW: I'm 31 years old. I was diagnosed at the age of two. I remember having multiple sinus surgeries when I was younger, but I've completely lost count. Since being Dr. Eskew's patient, I think he has performed five.

SM: I'm 38 years old. I was diagnosed at age 11 when they found polyps during surgery to remove my adenoids. In total, I've had probably around eight sinus surgeries, including the frontal obliteration and adenoids removal as a child.

2. When did you have a frontal obliteration? Have you had any sinus surgeries since the frontal obliteration?

BW: I had my obliteration on January 21, 2015. I have had one sinus surgery since then, a little over a year later.

SM: I had the frontal obliteration on December 14, 2011. I have had three or four surgeries since that time, as well as multiple in-office sinus washes and one in-office polyp removal.

DR. JAMES ESKEW AND BRITTANY WAGER.

3. What was the initial reasoning and/or determining circumstances that lead to scheduling this surgery as compared to regular sinus surgeries?

BW: I was having horrible headaches and horrible pressure in my face. I was admitted to the hospital and on various IV and oral antibiotics constantly. The year prior to my surgery, I was on antibiotics for a total of nine months out of the year. It was just kind of the next step that I knew I needed to take for myself. It seemed scary in the moments of talking about it, but Dr. Eskew made me feel so comfortable, and I completely trust this man with my life. I knew if he was recommending something this extreme, then it was probably for the best.

SM: I started having regular, debilitating headaches several years before I agreed to the surgery. I've had what I jokingly refer to as a full-time infection in my sinuses for the better part of a decade now. The various attempts to treat it with IV medication, oral antibiotics, sinus-nebulized antibiotics, office procedures, and routine sinus surgeries thus far hadn't provided any lasting results. The frontal obliteration seemed to be my last option for getting rid of all of the infection and hopefully my chronic headaches for good.

4. Was the surgery worth it? Was the outcome of the surgery worth the surgery and recovery time?

BW: I would say the surgery was worth it from a sinus stand point, and it has helped with lung infections as well. I'm not on antibiotics as much as I was before surgery, so the surgery was 100% worth it for me.

SM: That's such a tough question for me. If you gauge this based on the initial outcome of surgery, then no. Within a few months I was experiencing headaches again and culturing out the same sinus infection, albeit from different sinus cavities. Nearly eight

years later, I've learned to manage the sinus infection with routine visits to Dr. Eskew every three weeks for an in-office sinus rinse and with the addition of a compounded antifungal/antibiotic/steroid pill that I dissolve in my sinus rinse twice daily. All that aside, all of my pre-surgery scans showed what looked like bone in behind my eye and adjacent to the brain. When Dr. Eskew went in during surgery, he found that it wasn't just bone but a pocket of infection nearly encased by bone growth, which was dangerously close to rupturing or leaking, if you will, into either the eye socket or brain and ultimately would have resulted in blindness or meningitis. From this perspective alone, the surgery was 100% worth it and ultimately a life-saving procedure.

5. Speaking of recovery, how is or was that? Was it harder than expected? Was it longer than expected? What was the most surprising part of the entire recovery process? What worked best for you and what didn't work for you?

BW: The recovery was brutal. The days after, I can't even explain. It was the strangest recovery I have ever had. The most swelling to the face I ever have had. The months following the surgery were the hardest: I lost so much weight because I was so nauseated from all the headaches and vision issues (which were temporary). I still get really bad headaches, and my scar line is very, very tender. Even though before I was having daily headaches from sinus pressure, the headaches I have now are a totally different type of headache. I was told to expect a hard recovery, but I think the recovery was much harder than I had ever thought it was going to be. Even though the surgery was such a success for me, I honestly didn't expect to have issues four years later. The most surprising part of the entire recovery process was how amazing my sinuses felt afterwards. Instantly, I could smell things I hadn't smelled in years. My ability to taste changed. It was the greatest feeling. What worked best for me after surgery was to not push my body. I relaxed and let my body get the rest it needed to heal.

SM: Oh, gosh. Recovery for me was brutal. My head hurt so much immediately after surgery. The first few days in the hospital were such a challenge, physically and emotionally. The first 24 hours I was blindfolded with a drain tube snaking through my forehead to help keep the swelling around my eyes and in my head generally to a minimum. The immense amount of swelling in my head made sleeping incredibly difficult and nearly impossible, so much so that the pillow hurt my head. Before the surgery, my headaches were debilitating: I could no longer listen to music or read a book. Post-surgery, I felt like my head felt me back, which doesn't really make sense until you're acutely aware of your head and the tingling sensation when touched. There were big things and little things for me. After shaving off two inches from my hairline, Dr. Eskew applied a gel to keep the new hairline in place and avoid infection from stray hairs in the suture sites. This, plus seven hours of anesthesia, which comes out in your hair, created a horrendous, sticky, straw-like mess of hair that was extremely difficult to brush. After three months of both struggling to repair the damage to my existing hair and fussing over the shaved parts of my head, I decided to cut it all off into a very short pixie. Nerve endings were severed during surgery so little things like blow drying my hair resulted in unknowingly burning my scalp. I had to hold up my eyelid to apply eyeshadow as I couldn't really feel my eyelid, much less move it. As nerve endings regenerated, I experienced burning and itching sensations intermittently on my scalp. It was a full year before I recovered completely.

DR. JAMES ESKEW AND SYDNA MARSHALL.

6. What advice would you give to someone who is contemplating the surgery?

BW: The advice I would give is, if you are a cystic fibrosis patient you can do this! This is a funny question for me because I have had multiple people reach out to me throughout the years asking about this surgery and how it affected me and if I would do it again — the pros and cons. And I have always told everyone, yes, do this for your health. The pain after the surgery is temporary; it will subside. You have to look at your overall health: Will this benefit you? Could this possibly increase your lung functions and cause you to have fewer sinus surgeries in the future? Yes, the surgery itself is brutal and hard and it is not an easy recovery in the slightest bit, but it is a hurdle you are able to overcome and you will feel better once the worst part is over.

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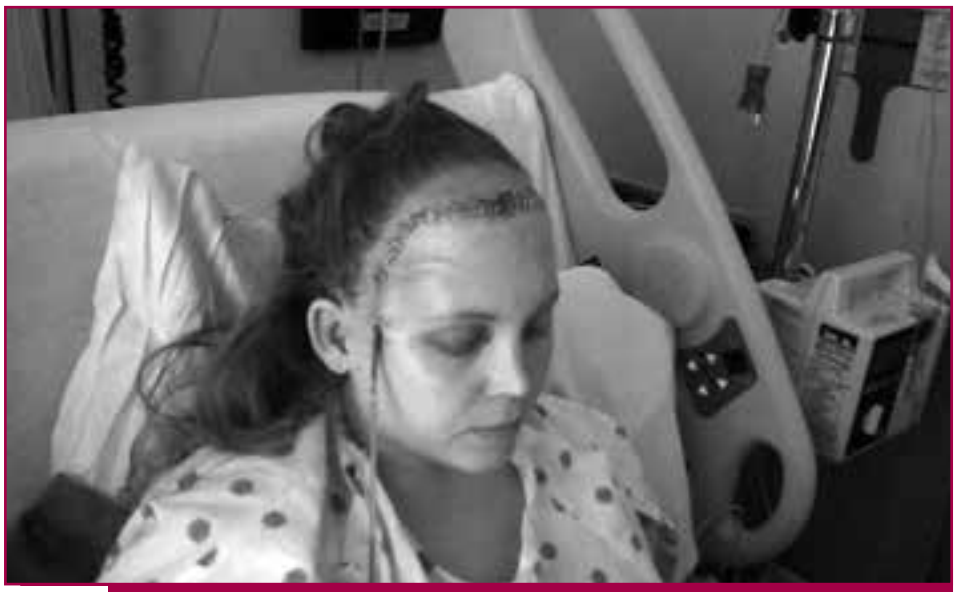
SM: I would say trust your gut and trust your doctor. It's a long procedure and it's a long year of recovery. For a long time I felt like I was trying to sugar coat a surgery that really didn't do what we'd hoped for in the beginning and then always fell back on the notion that it did save my life. Only in the last year have I truly accepted that my sinuses were and are always going to be terrible, so the best I can expect is managing them to be less debilitating day to day. Any expectations I used to have of complete recovery after undergoing maybe one or two sinus surgeries were unrealistic. Back in 2011, I hadn't fully understood nor accepted that the trajectory of sinus health was always sloping downward for me. In the end, I completely trust Dr. Eskew, and he felt so strongly that the frontal obliteration would be the solution to my recurrent sinus infections. Even though none of us could have predicted I'd be seeing him more now than I did back then, I don't regret trusting him. If you're considering one or have one scheduled, be patient. Recovery is long and slow. I had so much swelling that the pillow hurt my head, which really makes no sense unless you've had your head cut open.

7. What were some of your fears leading up to the surgery?

BW: I wouldn't really say I had fears leading up to the surgery. My main fear with surgeries is being put under anesthesia, but no other fears really crossed my mind. I talked with SM a lot before my surgery and she prepared me very well so I knew what to expect and how I was going to feel.

SM: I had so many fears. I didn't really have anyone to talk to other than Dr. Eskew, and the doctor's perspective on how it goes or how it might go with recovery is always different from the patient's perspective. I was worried about the vulnerability that comes from being blindfolded for 24 hours and having to use a bedpan and catheter. I was worried about whether my hair would come back different in color or style. I was worried about the pain level. I was worried that I might never fully recover. I was worried about so many things that weren't worth the stress!



PHOTOGRAPH OF SYDNA MARSHALL WAS TAKEN 12/15/11, ONE DAY POST-SURGERY, AFTER BLINDFOLD WAS REMOVED AND BEFORE DRAIN TUBE WAS REMOVED AT SETON HOSPITAL.

8. What does life look like post-surgery? How are your sinuses now compared to pre-surgery? What impact has this had on your sinuses and life, if any?

BW: My sinuses have been great compared to before. I still have pain years later, but they are different pains and I'm no longer having multiple sinus surgeries a year anymore.

SM: My sinuses now aren't really better, just different. The infection that originated in my forehead sinuses has moved to the maxillary (cheek) sinuses. I see Dr. Eskew every three weeks for an in-office scope and wash of my sinuses. Despite being colonized by the same infection day in and day out, I no longer have debilitating headaches. I suspect that because we're able to clean out my sinuses, as opposed to letting the infection fester and grow, I don't suffer from the swelling and side effects nearly as much as I did before my frontal obliteration. The addition of a compounded antibiotic/steroid/antifungal in my daily sinus rinses has helped with the swelling tremendously. Seeing Dr. Eskew as often as I do and no longer working have really made the biggest impact on both my sinuses and the time between my hospital admissions. ▲

Sydna is 38 and has CF. She is a native of Austin, TX. She lives with her husband, Adam, and fur baby, Husker. She's an avid reader, yogi, and part-time gourmet foodie. You can generally find her nose in a book when she's not enjoying all that Austin has to offer. She is a director and Secretary of USACFA. Her contact information is on page 2.

Brittany is 31 and has CF. She is a native Texan. She lives with her husband, Joseph, and four fur babies, Rosie, Duke, Dori, and Kitty, in Texas hill country. She loves baking, crocheting and blogging about her life experiences. When she isn't working on a project, you can find her exploring the hill country with Joseph.

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

Ella Balasa
Richmond, VA
27 on May 2, 2019

Jeanie Hanley
Los Angeles, CA
57 on June 12, 2019

Aimee LeCointre
Salt Lake City, UT
34 on April 20, 2019

Laura Mentch
Bozeman, MT
66 on May 21, 2019

Kathy Russell
Gresham, OR
75 on April 17, 2019

Mark Tremblay
Albany, NY
50 on June 6, 2019

Wedding

Kathy & Paul Russell
Gresham, OR
54 years on March 27, 2019

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New Strategy May Boost Antibiotics' Efficacy In CF Patients, Study Finds

Removing a key metabolite called pyruvate from Pseudomonas aeruginosa and Staphylococcus aureus bacteria biofilms could help boost the effectiveness of antibiotics. Disrupting biofilms could represent an easier and more effective treatment for such infections.

Because pyruvate, the end product of a metabolic cellular process called glycolysis, can impair the initial formation of biofilms, researchers decided to test whether removing pyruvate would have an impact on biofilm maintenance and dispersion of bacteria.

Bacteria surface attachment is a multi-step process where the final step, called dispersion, is a regulated process by which bacteria escape biofilm status, and transition to the free-living mode of growth — known as planktonic mode. To test their hypothesis, researchers used an enzyme called pyruvate dehydrogenase (PDH) that degrades pyruvate into other molecules. They observed that P. aeruginosa bacteria in biofilms treated with PDH were reduced by about 794 times with increasing concentrations of the enzyme. A similar observation was made with S. aureus biofilms. Because previous studies showed that bacteria in the dispersion phase are more susceptible to antimicrobial action, the researchers then treated P. aeruginosa biofilms with the antibiotic tobramycin, both in the absence and presence of PDH. Results showed that treatment with tobramycin together with PDH improved antibiotic efficiency (251 times more efficient) than the antibiotic alone.
https://tinyurl.com/y3nyo44e

Perth-based Biotech Company Drug Breathes Life Into Kids With Cystic Fibrosis

A new biotech company could deliver a major breakthrough in the treatment of cystic fibrosis (CF) using tweaked antibiotics to reduce damaging lung infections.

Instead of focusing on genetic abnormalities that cause CF, researchers are using a new inhaled drug to target antibiotic-resistant infections that wear down the lungs and restrict breathing. The therapy uses the antibiotic tobramycin with an additive that helps break down the biofilms that bacteria build to protect themselves in the lungs.
https://tinyurl.com/yxgoajxc

Dual β-Lactam Combinations Highly Active Against Mycobacterium Abscessus Complex In Vitro

There has been a dramatic increase in chronic infections caused by Mycobacterium abscessus complex (MABC) strains that are usually recalcitrant to effective antibiotic therapy. The recent rise of macrolide resistance in MABC has further complicated this clinical dilemma, dramatizing the need for novel agents. The repurposing of current antibiotics is one rapid path from discovery to patient care. In this study, researchers discovered that dual β-lactams, and specifically the combination of ceftazidime with either ceftaroline or imipenem, are synergistic and have clinically relevant activities against clinical MABC isolates.

Continued on page 18

Sixth Time The Charm?

By Andrea Eisenman

It was the end of a lousy health year. I had many health issues because I needed to be on Rituxan, an immune-therapy/chemotherapy drug, in order to fight post-lung transplant lymphoproliferative disease (PTLD). I then needed to recover from pancreatitis that lasted two months. My lungs were compromised due to my sinuses draining into my lungs, congesting them, and I was coughing up infected mucus. I knew I needed sinus surgery, my sixth, but I didn't have great hope that the surgery would provide incredible relief for very long.

Prior to this surgery, I had five sinus surgeries with varying degrees of success. I was told that this time would be the game changer. Why? Answer: A new doctor and a new procedure. He was not only going to knock down the walls in my sinuses but also would remove my turbinates. He wanted to make a bigger space and hopefully get better drainage. Post-surgery, I would

ANDREA EISENMAN

use antibiotics mixed with a pre-mixed packet to flush my sinuses and keep them clear. The combination of two antibiotics and one anti-inflammatory agent would be based on my cultures during surgery.

It was to be a New Year's Special! That was the earliest date the ENT was available, or I would have had to wait until March. I agreed as I am not a big New Year's Eve person and, this year, I figured I would be stoned out of my mind with opiates to minimize my pain (or so I thought). It was weird to be in the recovery room with only a handful of other patients, and I just hoped that those nurses still on duty were not dipping into the spiked egg nog too early in the evening.

To my surprise, the pain was pretty minimal when I left the hospital. Although I was draining a river from my nose, I was not in pain. When I had visited the doctor in his office, he had scared me with the information that he would be removing my turbinates, which I was advised could be painful (later, I also found out he had removed part of my upper jaw).

Why remove the turbinates? That allows a larger cavity and better drainage.

TILLMAN continued from page 17

https://tinyurl.com/y4wtza8c

Preventing "Cell Wall Remodeling" May Hold Key To Defeating Intransigent Super Bugs In Cystic Fibrosis, Other Diseases

B. multivorans is a notorious pathogen that can cause infections such as pneumonia in immune-compromised individuals with underlying lung diseases. This pathogen can also cause rapid clinical deterioration in patients, including blood stream infections, which can lead to death. What makes B. multivorans especially dangerous is that it is intrinsically resistant to a broad range of antibiotics, creating a major challenge to treatment.

Researchers will focus on describing the structure and function of cell-wall strengthening ("remodeling") machinery in B. multivorans and identifying peptide-based inhibitors to block this process, thus reducing the potential life-threatening activity of the bacteria. Previously, it was discovered that B. multivorans uses reinforced cell walls to thwart antibiotics. It does this by transporting lipid compounds – called hopanoids – from its inner cell membrane to its outer cell membrane, making the latter stronger and stiffer. This prevents antibiotics from doing their job by rendering the bacteria impregnable.

The researchers will target two proteins called HpnN and HpnM. The team will examine both proteins to determine their precise role in strengthening the outer membrane and thus prohibiting antibiotics from entering and doing their job.

https://tinyurl.com/y4ch4xpb

Targeting Bacteria Cell-Wall Elements Can Help Fight P. Aeruginosa

Targeting some elements in the bacterial cell wall can be an effective way to activate the immune system and fight chronic, treatment-resistant Pseudomonas aeruginosa infections, according to a study. Human immune cells natur-

To be done ONLY if a patient has already lost their sense of smell — which for me was the case several years ago. Since that was not a worry, we proceeded with this surgery plan. And, I prepared to be on heavy pain medications. Thankfully, I didn't need them!

The surgeon told my husband that, during surgery, he saw a huge amount of infection and inflammation and whipped up an antibiotic paste that he applied directly to the affected areas. He was truly amazed I was functioning at all with what was going on in there. To compound it, these infections were plaguing me, causing me to go on heavy IV antibiotics twice a year to keep my transplanted lungs functioning at their peak. It seemed it was imperative to get my sinuses in better condition. My PFTs had been dropping, and I was short of breath at times, which was unusual for me post-lung transplant.

The surgeon told me that he had put sponges in my nasal cavities to keep those areas open, but I was to start the nasal rinses a day after the surgery. I could immediately feel the difference. I was breathing in much deeper and with less pressure in my face and cheeks. Then, once I saw him about a week later, he removed the sponges and WOW! It was amazing how much more cavernous it felt in my sinuses. Then, each time I saw him, he removed scabs and used suction to remove mucus. After each visit, I was shocked at how much better I was feeling and breathing. My eyes, which had been puffy and swollen for years, started to return to normal. I didn't have headaches and sinus pressure, no invisible vice tightening around my head. My lungs were less affected by post-nasal drip and, therefore, less congested.

I was exuberant. But I was wary as this was now my sixth surgery in 19 years. Would it hold? I am not sure yet. But the antibiotic flushes are helpful, and I now use a squeeze bottle instead of a Neti pot. I get more force that way and I see more of the gunk flushing out.

It is probably too soon for me to do a victory dance. A few weeks ago, I got very nervous when allergy season started, I immediately became congested nasally and had copious, thick, dark discharge coming from my nose. I called the ENT in a panic and he suggested I wait a few days before doing IVs, knowing it was probably a reaction to the pollen. He was so right. In two days, during which I religiously flushed my sinuses with medicated rinses, my mucus lightened and so did the pressure.

While surgery is never to be taken lightly, those with diseased sinuses are old pros with this surgery, many undergoing it once to numerous times. I was scared this time because it was a new surgeon and going under general anesthesia can be risky. But I am so glad I had this procedure. It has definitely made a difference in my overall health. ▲

Andrea is 54 and has CF. She and her husband, Steve, live in New York, NY, with their dogs. She is a director of USACFA and the Executive Editor of CF Roundtable. *Her contact information is on page 2.*

ally produce proteins to target important bacteria cell wall elements, called peptidoglycans (PGNs). With this strategy, the immune system can block some of the natural protective mechanisms of bacteria. Researchers have now evaluated the antibacterial potential of these immune-produced proteins — namely lysozyme and PGN Recognition Proteins (PGLYRPs) — to fight treatment-resistant infections. The team exposed bacteria strains to immune-produced lysozyme or PGLYRPs. Overall, results showed that the antibacterial activity of these proteins alone was modest for the majority of the strains. Interestingly, when the team used either lysozyme or PGLYRPs in combination with a permeabilizer compound (subinhibitory colistin — a compound that disrupts the permeability of the bacterial cell wall), the antibacterial activity was enhanced. The increased bacterial susceptibility to the combo treatment could result from particular changes in PNGs and their metabolism. On the other hand, niche adaptation in the case of CF could also contribute to bacteria cell-wall protein changes, and consequent responsiveness to treatment. According to the team, the results suggest that attacking some P. aeruginosa cell-wall biology-related elements to increase the activity of our innate weapons could be a promising therapeutic strategy.
https://tinyurl.com/yy8fhpdq

In CF, Specific Peptide May Lower Bacteria's Antibiotic Resistance, Study Says

Expression of a peptide called RpoN* in antibiotic-resistant Pseudomonas aeruginosa isolates from cystic fibrosis (CF) patients made the bacteria more susceptible to antibiotics.

RNA polymerase, nitrogen-limitation N (RpoN) is a sigma factor — a protein needed to start bacterial DNA transcription (the process of copying a gene's DNA sequence to make an RNA

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Can't You Smell The Skunk?

By Katie Lockwood

"Close the window...Katie...close the window!" said my husband as we drove down a Cape Cod country road. "It's a skunk! Can't you smell it?"

"No, I can't," I said. "Well, if I try... maybe a little. Not enough to freak out about a window being open."

Sometimes I even pretend to smell things. It's easier than explaining why I cannot. Smell this, Katie. Isn't it amazing...Yes, it's wonderful. I am sure.

The truth is, I have cystic fibrosis and I haven't had a good sense of smell or a normal sense of taste for most of my life. Throughout my life, I have begun my morning by coughing up all the post-nasal drip that accumulated during the night. I keep a stockpile of tissues to blow my nose and good tea to soothe my throat, including Throat Coat and Cold Care by Traditional Medicinals and Hot Cinnamon Spice by Harney and Sons. Highly recommended!

My first surgery was a few years prior to the other one and was somewhat helpful in reducing my headaches...for a while. After the second sinus surgery, there were six months or so after they removed 20 or so grape-sized polyps from my sinus cavities when I could smell and taste everything. Since the level of smells and tastes were new to me, it was overwhelming! My favorite food is any kind of Chinese food and for those six months, I found the tastes overpowering.

As someone with relatively mild lung issues, my sinuses are one of my biggest headaches. While I may have been slightly relieved when the benefits of surgery wore off and I could go back to enjoying my Chinese combo plates, the sinus pressure and the pain returned as well. I find some sinus relief by taking 600mg ibuprofen and doing daily sinus rinses with a Neti pot with a saline solution. While I am probably overdue for another surgery, I didn't find it especially helpful and anesthesia makes me anxious so I plan on holding off if possible. It is impossible to fully separate out CF symptoms, but I prioritize my self-care.

KATIE LOCKWOOD

For me that takes the form of taking a bath every evening to decompress, reading daily, getting a $35, half-hour massage weekly (includes facial massage), a minimum of eight hours sleep, and I visit with family and friends weekly. I am very excited for the new CFTR modulators from Vertex to become available. In addition to the wonderful opportunities for PFTs, there is also the potential for this medication to lessen sinus symptoms, which would be awesome! ▲

Katie is 31 years old and has CF. She lives with her husband, Arden, and their cat, Panji, on Cape Cod, MA. They enjoy spending time on the water, traveling, champagne toasts, and Chinese combo plates. You can follow her website at https://sweetyetsalty.weebly.com. Katie and Arden are currently trying to conceive via IV, and she is capturing their story through @chronicallyillfinanciallyfit on Instagram.

USACFA Speakers Bureau

The U.S. Adult CF Association, Inc. (USACFA) now has speakers who will come to speak at fundraisers, education days, and other CF-related events. All the speakers are adults who have CF and can speak with experience on living with CF and what is happening in the CF world. USACFA has budgeted for travel and lodging costs for the speakers, so there is no cost to the hosting organization.

If your organization is interested in having a CF speaker present at your event, please contact speakers-bureau@cfroundtable.com

PHOTO BY PHILLIP HOWELL

Tachypnea

Hummingbird,
diminutive and fierce.
My breath comes as quickly
as the beating of your wings.
My hunger for sustenance
will match your voracious appetite.
My spirit wills itself
to adopt your tenacity.
Swooping
Darting
Intently surviving.

- C. Martinet, 1999

FROM OUR FAMILY PHOTO ALBUM...

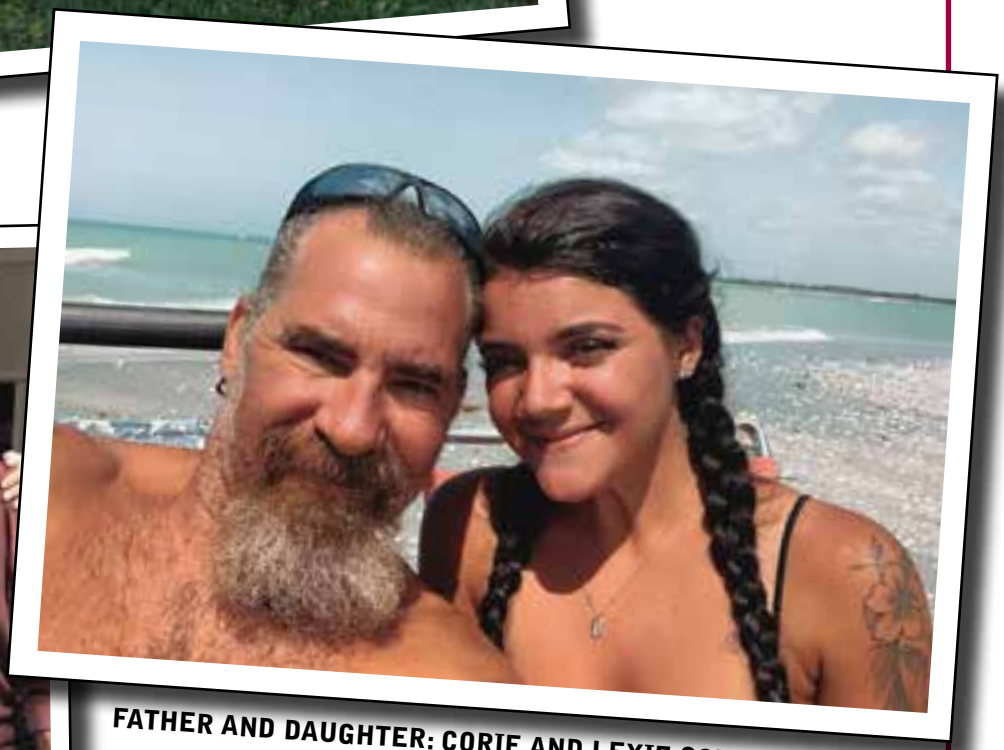
JEANIE HANLEY AND HER DAUGHTER, MARIA HENDRICKSON, IN JUNE 2019, AT WASHINGTON, D.C., SMITHSONIAN GARDENS.

REBECCA MUELLER

ANNUAL COUSIN WEEKEND IN VERMONT WITH: FRIEND WENDY JAMSRI, BROTHER MICHAEL LOW, KATIE LOCKWOOD AND HER HUSBAND, ARDEN, AND COUSINS TOM AND MILLY SEJKORA.

TRÉ LA ROSA WITH HIS DOG DUNCAN.

WIFE MARYGRACE AND MARK TREMBLAY AT THE FINISH LINE OF THE LOCAL CF CYCLE FOR LIFE IN 2018.



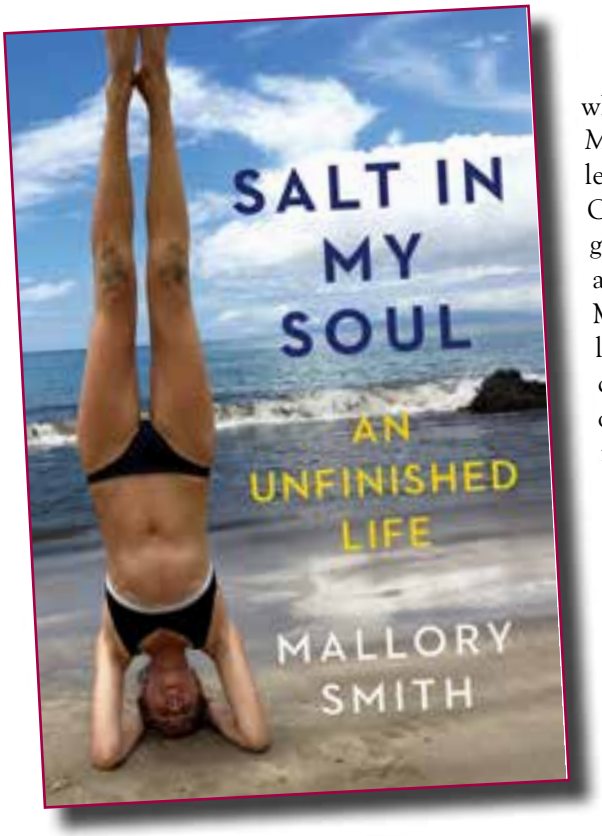
FATHER AND DAUGHTER: CORIE AND LEXIE SCHULLER.

ANDREA EISENMAN WITH HER CHILDHOOD FRIEND KERRY MONCURE (AND HER HUSBAND, JEFF SMITH).

BOOK REVIEW

Salt in My Soul: An Unfinished Life

(New York: Spiegel and Grau, 2019). 303 pp.
By Mallory Smith.
Reviewed by Rob De La Noval



Salt in My Soul is not an easy book. Like every story of a life authentically lived, it is an alternation of joy and sorrow, expectation, and despair. It is, to borrow the title of another bestseller of years past, a book about the audacity of hope. How can one hope in the face of an inevitably terminal illness? Is such hope a necessary fiction that helps in the struggle of doing the day's work, a sort of optical illusion self-induced to prevent us from giving up prematurely? But what can "prematurely" even mean when the end is already living inside us, in our most intimate of organs, the channel of life's breath, consuming us from within? For CF is living death; CF is deathly living; CF is death and life interwoven — clasping, grappling, contending.

It is this question of hope that animates the entirety of Mallory Smith's life, and it is a question that anyone with CF will recognize as the central existential challenge of this disease. But, as Mallory so well understood, it is also the question put to any human spirit who will grace this earth for any period of time: "The question of how to meaningfully spend a life is not unique to those of us with health challenges. But for people with cystic fibrosis, or stage IV lung cancer, or any other life-limiting illness, there is a certainty that life will most likely be cut short to some extent. This certainty forces us to examine our values, prioritize our time, and search for meaning now rather than later" (185). CF as a crystallization of the human condition, as a window into the pain and suffering that all people know in their most intimate depths — this was the secret Mallory discovered, one that we learn along with her in the almost ten years these journals cover, from her high school days to her heart-rending death in young adulthood. "[CF] has given me the mountain that's been waiting for me all my life. The mountain we're all climbing, every day. It looks different for everyone, but we all have our own struggles, every person I see on the street. I have to remind myself not to envy those whose lives look normal, because their mountains do exist, even if they're less obvious than mine" (xiv).

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As this quote testifies, what slowly unfolds in *Salt in My Soul* is the growth of a soul learning self-transcendence. Concomitant with her struggle to hope in the face of almost certain loss was Mallory's confidence that life's time was given her precisely for the flourishing of others, for shared communion. She learned this truth in her childhood, from her family, especially her mother and father and brother, equally amazing characters in this story, who can be described no more perfectly than with the adjectives "always present" and "self-sacrificial." The joy in Mallory's soul we repeatedly encounter in the journals is joy with others, whether those others be her dear family or her beloved friends in her college years, or even — what is especially remarkable — her joy in this earth as it motivated her writing advocacy projects in post-graduate life. Her love for the planet, its history, its progression, its mystery, all shine a unique light on this woman who easily could have held a grudge against this world and its torturous evolution, producing, as it has, such vicious legions of disease and death as CF. But her overriding attitude to the planet's history and her own personal intersection with it was one of gratitude and wonder: "My life is a miracle. Life in general is a miracle" (111).

In the journals we discover the moral pathos of Mallory's continual struggle between desiring the good for the whole and the good for herself as

an individual. This contest plays out not only in her relationship with the earth, in her occasional ethically motivated vegetarianism, and in her desire to pass on genes the evolutionary process would consider "imperfect." It also manifests in her continual awareness of, and care for, the well-being of those around her. We read of her gratitude toward healthcare workers and her patience and compassion for those whose ignorance of CF causes them to treat her unjustly. We marvel at her solicitousness for the emotional lives of her parents, of the state of their marriage in the context of her illness, of how they are managing their grief over the deaths of their own parents. One of the greatest terrors that self-conscious CF patients experience is the fear of becoming trapped within the horizon of their own suffering; Mallory deftly avoids this snare with an almost preternatural ability to love, to care, to keep others in her heart.

If Mallory's story says anything to CF patients in particular — and it says many things — it compellingly and movingly communicates the value of calculated risk-taking in a CF life. No choices are ever made with perfect knowledge of all variables and contingencies, and so the possibilities that so often present themselves to our healthier peers as pure opportunity appear to us as traps in disguise, as well as future sources of regret. This makes it all the more remarkable when we find Mallory, after agonizing over whether her time at college negatively and permanently impacted her health, coming to the conclusion that life is about quality, not quantity. "I sacrificed my health to go to Stanford — maybe I would have declined no matter what, but I definitely declined more by being away at college. It was worth it, 100 percent worth it, I wouldn't go back and change a thing, except maybe I would delete my period of depression" (140). So often have I *post factum* berated myself for ventures and adventures that came with unexpected health repercussions; sharing in Mallory's own self-questioning and, in her ultimate affirmation of the value of embracing thoughtful risk, I find some solace that I am not alone.

These mental and emotional exercises in second-guessing our choices point to another key value of the book for those with CF: its clear depiction of the mental health struggles built into this devastating illness. To name just a few: the constant shifting of goal posts as our health outlook improves or reverses; the unceasing, low-grade anxiety of the "ticking time bomb" of hemoptysis ready to explode at any moment; the responsibilities of daily adult life packed on top of the complicated medical regimens we need to keep us going; the challenge of being one's own advocate toward medical professionals and occasionally morally corrupt insurance companies; the need to make weighty medical decisions in the face of conflicting expert advice; the navigation of family relationships and natural growing pains made more complex by CF; the fears of rejection in intimate romantic relationships; the burden of being an inspiration to those who would consume our life stories in a superficial manner; the terror of watching others with CF reach the end of their journeys before we do, often in tragedy, and wondering how long we have until we fill their shoes and become a spectacle of suffering for the CF patients coming behind us. All these realities are endemic to a life with CF, and no one can bear them alone. Mallory's incredible community and resilient spirit helped keep her afloat, but her recognition near the end of her life of a need for psychological therapy reminds those of us with CF to seek professional help as we navigate this path strewn with physical obstacles and emotional land mines.

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You may know of the Catholic practice of venerating saints. In that tradition (which is my own), saints are people who have lived their lives with such intensity and transparency to Ultimate Reality — what believers call God — that their lives become examples to follow as we make our own journey through the wilderness of existence, so terrifying and so unknown. How can we live in love toward others and toward life itself in the face of so much suffering, bewilderment, and disappointing heartbreak? We need guideposts. Saints, with their lives of self-sacrifice, devotion, and care for others, mark a path for us, teaching us and encouraging us; they don't impart a message so much as they themselves, through their lives, *become* the message.

I thought of the saints as I read

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> "It is this question of hope that animates the entirety of Mallory Smith's life, and it is a question that anyone with CF will recognize as the central existential challenge of this disease."

Meet A New Director - Tré La Rosa

I am a 25-year-old adult with cystic fibrosis living in the Cincinnati, OH, suburbs. I'm an alumnus of the University of Kentucky, where I studied biochemistry and minored in mathematics and biology. After graduating, I was fortunate enough to find a lab at Cincinnati Children's Hospital that was doing compelling research on personalized medicine, mostly focusing on modulators and patients with rare diseases. Working in the lab has been both poetic and profound: I was treated at Cincinnati Children's for 12 years, so working behind the scenes has been especially rewarding. While working alongside brilliant scientists and doctors, it's been a great privilege to see the scientific innovations happening in the CF world.

After graduating college, I started writing because I realized the benefits it provided: a chance to tell my story, thus increasing awareness — but it is also therapeutic for me. Hopefully, it also helps others to be open about their lives, whether it's about mental health, grief, or a fascination with a niche topic. I have made it a priority to become a professional advocate and consider it my biggest goal to ensure that I'm always including as many voices as possible. I hope my experience on the Blog committee will advance the conversation around CF — in medicine, science, and society.

TRÉ LA ROSA

My health has been stable for several years, and I'm very fortunate to have had a positive response to Orkambi (I'm a ΔF508 homozygote). My sister's health, on the other hand, was not so fortunate: she passed away due to chronic rejection of her second bilateral lung transplant in March of 2018. My sister was a huge role model in my life. Her spirit, her perseverance, the way she treated others always inspired me. It breaks my heart that she was unable to maintain a full-time job, get married, or even move out on her own. Witnessing her experiences certainly helped me become more attuned to how difficult it is for people with chronic diseases and disabilities to live fulfilled and independent lives. ▲

DE LA NOVAL continued from page 25

Mallory Smith's journals in *Salt in My Soul*. Her remarkable story, so vulnerably on display in these pages, reminded me of the many saints' lives I have read, stories lovingly transmitted from generation to generation by those touched and *changed* by them. Every year in the CF community we learn the tragic news of the beloved people this disease has taken from us. They pass on, leaving behind an imprint of love on the hearts of those near them, and their memory emerges suddenly as a vulnerable treasure to be cherished and kept, cultivated and handed on. To that end of preserving a life in the heart's memory, few means are more powerful than the written word. It is our good fortune that the written word was Mallory's exquisite gift, and that her family saw fit to share her life with us so that her wisdom might spread, and that she might live on in the memory of hearts she never knew but will nonetheless intimately befriend the moment her words are read.

I conclude by noting that this extraordinary memoir bears the subtitle "An Unfinished Life." While such a description is eminently understandable from the perspective of those who knew Mallory and loved her, of those whose lives are irrevocably altered by her death, I wonder whether another subtitle might have been equally apt: "A Perfected Life." In the Greek language, the word for "end," *telos*, means both "end" and "goal" — to reach one's end is to reach the purpose, the aim, the reason one set out on a journey in the first place. In spite of all the beautiful things in life her CF took away from her, Mallory nonetheless felt she had achieved something precisely *through* her CF: "I feel like people with CF are privy to secrets it takes most other people a lifetime to understand. How lucky we are to be alive. How lucky anyone is who has their health. How we should be appreciative of anything that's in our control since our health is not. That we can leave behind a legacy when we go that will impact others. That simple things are often

Meet A New Director - Mark Tremblay

My name is Mark Tremblay. I am 50 years old and have CF. I was diagnosed in 1970. I am psyched to be a member of the USACFA board, which will give me a chance to share my experience, work with other dedicated CF professionals, and learn from the observations of our broader CF community. I look forward to raising awareness and providing encouragement to other CF patients, families, loved ones, and care providers — particularly in the areas of psychological, emotional, and social well-being.

In addition to having CF, I'm 32 years sober. As an adolescent, I drank heavily and used drugs to deal with the emotional and psychological burden of CF, until I reached the age of 18. In a few short years, I went from being a patient in recovery to being an administrator for a drug and alcohol treatment center in Syracuse, NY.

Sobriety gave me the strength to face my disease head on. During that time, I was very active in the CF community: participating in CF Foundation events, fundraising, public speaking, writing articles for the International Adult Cystic Fibrosis Foundation Newsletter, as well as making radio and television appearances. As an undergraduate and graduate psychology student at Le Moyne College and Marywood University, I researched psychological and emotional well-being in CF patients. In fact, my master's thesis became a multi-year study, which was published and became an abstract at the American Thoracic Society Conference in 1999.

MARK AND MARYGRACE TREMBLAY

However, after having a major exacerbation in my late 20s, I left all that behind to study health policy. I did that by pursuing an MPA at Syracuse University Maxwell School of Citizenship and Public Affairs, which led to a 20-year career with the New York State Division of Budget and Department of Health. Currently, I'm working full time, living with my wife, MaryGrace, of six years, and my stepson, Sean, in Albany, NY. My wife and I love road cycling, motorcycling, traveling, spending time with our families, and volunteering at church, including helping to lead the Celebrate Recovery ministry. ▲

the most beautiful. That love and happiness are the most important things to strive for...CF has given me my value system and ultimately, no matter how hard it is, I'm grateful for it" (236). Mallory's life as told in *Salt in My Soul* was a life replete with love, as saturated by it as her soul was with the salt of suffering. Only such a soul could say with sincerity that the love of her family had taught her to feel, despite her illness, that "the world is a good place" (294). That love could make this vale of tears we inhabit a good place is the most realistic of hopes for enduring the unpredictable and ruthless sufferings of life; whatever our circumstances, and however much time we may have, we are always being gifted with the possibility of allowing love at work in us to make others feel that life is worth the living. Any who read Mallory's story, told here in her precious words, will learn something of how to live with that hope, so that no matter how their days may end, ripened or premature, theirs will be a life unfinished only in time, not in love. ▲

> "In the journals we discover the moral pathos of Mallory's continual struggle between desiring the good for the whole and the good for herself as an individual."

Rob De La Noval, is 30 and has CF. He lives in South Bend, IN. He is a doctoral candidate in theology at the University of Notre Dame. He can be reached at robertdln@gmail.com.

There Is Nothing Stopping Me

By Alexis Schuller

> "Lately, this disease has set a fire under me to go out and get everything I have always wanted."

When I was nine years old, a baby girl was welcomed into my family. After newborn tests had been performed on her, she tested positive for cystic fibrosis. My family was not familiar with the disease, so they began reading up on it and instantly realized I had it as well. I had been shuffled to various doctors my entire life, while my parents tried to figure out what was wrong with me and, just like that, my sister came into the world and the mystery was solved.

Finding out you have a serious disease at such a young age can be very emotional and confusing. All of a sudden, I had to take handfuls of pills throughout the day, be on a breathing treatment regimen, and endure frequent visits to doctors. It is very tough to make the transition from a "normal life" to having CF.

From the day I was diagnosed, I was very verbal about having CF. Almost everyone at school and at my various clubs knew I had it. I would try to do some sort of fundraiser each year to help raise money for a cure. My dad has always instilled in me that having this disease does not give me an excuse or a free pass. Since elementary school, I have always had stellar grades and I even graduated high school in the top ten in my class. I am currently studying business administration with a concentration in finance, and I have maintained a 4.0 GPA the entire time. But I have also gone through some rough patches that partly stemmed from my CF.

When I was a freshman in high school, I came down with a double ear infection and antibiotics were just not doing the trick. After an excruciating night of pain, both of my ear drums ruptured. I was rushed to the hospital and spent nearly three weeks in a hospital bed, experiencing the worst pain of my life. When I was first admitted, I was so worried about my school work, that my guidance counselor had to call me and reassure me that I would get to retake my midterms and that my only job was to focus on getting better. While I was in the hospital, the infection spread to the bones in the back of my head, and I ended up being homeschooled for the next three months. I missed almost four months of school during my freshman year. It was hands down the scariest time of my life. The amount of pain I was in seemed inhuman and, at the time, I was almost sure I was dying. I was, however, pretty healthy the rest of my high school career and was not hospitalized again. When it came time to think about college, I applied to six schools and got accepted into all of them, some on full scholarship. I turned them all down, though, and registered for community college. My family was worried about me going far away and not being able to take care of myself; if something were to happen to me, they would be many miles away.

ALEXIS SCHULLER

On my first day of college, I freaked out and dropped all of my classes. What was I going to do if I got sick like I had been just a few years ago? Colleges do not have the same 504 plans in place that grade and high schools have. There was no way I would be able to pass my classes on my own if I were to get hospitalized. I was in a rough place that whole year, just working and without direction. My dad kept trying to encourage me to go back to school and at least attain my associate's degree. But life was starting to scare me because, for the first time, I considered the average life expectancy for people with CF. The thought of not making it past my thirties alarmed me. Why was I going to waste four years in school to prepare for a career that I would maybe work in for five years before I got too sick to work? I hated the world and could not get over how unfair I thought it was.

I could not tell you what changed but, one day, I woke up and told my dad I was reapplying for college. He was so excited. Although he was in the middle of work, he stopped everything he was doing and made me apply at that very moment. I have always been very ambitious and had so many goals for myself, and I decided that I was not going to let some disease dictate what I do with my

life. Lately, this disease has set a fire under me to go out and get everything I have always wanted. I have roughly two years left until I am done with my bachelor's degree, and I am in the process of trying to start my own business. I hope to one day be able to travel and speak about my story and help influence others who may have a life-pressing medical circumstance like CF. I also would like to own multiple businesses, especially starting one where I can work alongside my dad and brother each day.

CF can throw a wrench in your life plans, so you just need to be prepared for it. I know it would not be optimal for me to work a nine-to-five job every day under somebody else because, in the event that I get seriously ill, I do not want to have to worry about losing out on promotions or even being replaced. By owning my own business, I will have the freedom to make my own hours and, if I am not feeling myself, I have the freedom to take the day off without facing repercussions. It also is something I can pass down to my future children or family so I would know they would have no financial struggles once my time here runs out.

One time, I told someone I had CF and they asked me why I bother going to school or working, suggesting that I should just try to get on disability. I just remember how shocked I was that someone could have that mindset. I am beyond grateful for the fact that my body is letting me work and go to school, and I am going to keep going until it does not let me anymore. I have so much drive to achieve everything I have ever wanted out of this life and you'd have to be crazy to think a disease is going to stop me. ▲

Alexis, or Lex, is 20 years old and has CF. She lives in Fort Myers, Florida. When she's not working or busy with school work, you can catch her soaking up the sun with friends or family. You can contact her via e-mail at lexieschuller98@gmail.com.

IN THE SPOTLIGHT

With Rebecca Mueller

By Andrea Eisenman and Jeanie Hanley

For her dissertation, Rebecca Mueller was conducting a study on cross-infection in regards to the CF community. She asked me, Andrea, about taking part in it. I was happy to participate, to further the understanding of the ramifications for people with CF on whether they are able to socialize with one another. My doctor frowned upon people with CF being in the same vicinity as each other. I remember how lonely I felt not knowing others with CF, and I had little peer support of people who really understood. I was impressed and intrigued with Roberta's work in this area, sure it would help others in the future. I had hoped that after I took part in her study, she would volunteer to answer my questions for an interview for *In The Spotlight.* She agreed, much to my delight. Meet our newest star. Spotlight, please!

Age: 37

Where do you live?

I live in Philadelphia and I grew up here. It is home. I love the scale of the city and the few degrees of separation between everyone. All the chapters of my life have been here, aside from college, so I have social circles from each phase of life that I value deeply.

Explain how you chose this career path:

In college, I gravitated towards anthropological studies of illness, designing a major within the American Studies program. My academic studies in college were vital for providing me with new ways to understand my experience with CF. They also piqued an interest in diabetes, given its chronic nature and pediatric onset for type 1. In college, I was fortunate to get a research coordinator job where I coordinated clinical trials of new therapies for people with type 1 diabetes. Working with patients was interesting as was the exposure to genetic counseling, since we hoped to integrate some genetic testing into our research protocol. My late stepdad was a geneticist and our relationship was formative in directing me to my career in genetic counseling. In my experience with CF, I find the data to be almost as hard as the bodily experience. Whether discussing my genotype (double delta F508, classical CF), my lung function, or dexa-scan, I find much medical information discouraging and wish that my providers were sensitive to this. Becoming a genetic counselor meant that I could help patients adapt to, and understand, many aspects of the risks of genetic diseases and the diseases themselves. I also was able to provide counseling and resources to aid in adjustment and family communication.

REBECCA MEULLER LEARNING TO SAIL, OCTOBER 2018.

What do you do now?

After working as a genetic counselor for three years, I returned to my first love of studying medicine from the perspective of the social sciences. I am a funded doctoral student studying the medical humanities under a mentor whom I met at a CF conference. She was presenting on the history of CF and I was speaking from the perspective of a patient. My dissertation explores cross-infection risk and the reconfiguration of the CF community in light of it. I am working to characterize the experience of the CF community before and after cross-infection concerns. Though I knew one person with CF growing up and had one hospitalization where we were still allowed to be social, I have not been immersed in a CF community since forays on Cystic-L online in my early 20s. Reading archived Cystic-L posts from the 1990s is what cemented my interest in this topic as the posts about the closure of camps were so heartfelt. In the course of my research, it has been a pleasure to talk to other people with CF and to

hear how people with CF think about the risks and benefits of socializing on- and offline.

How is your health now?

My health feels in transition right now. I find that the littlest things like rhinovirus or exposure to fumes irritate my lungs and that any kind of infection can cause an exacerbation, which was not the case before. I feel I am holding steady but with greater effort than in the past. Exercise is what I believe sustains me and maintains my current function. I can only hope that the new triple combination therapies help to stabilize me or, at least, give me a bump.

> *"Exercise is what I believe sustains me and maintains my current function."*

Being a double delta F508, are you on any new corrector/modulator drugs and are they helping?

Yes, I am on Symdeko. I felt clinically better after being on it for a few months and am stable compared to before, but I have not seen an improvement in terms of metrics. I have also simultaneously done many things to stabilize my health in this period, such as increased exercise, better hydration and rest, Pulmozyme, and different inhaled antibiotics.

What do you do for exercise?

My exercise regimen involves three-mile runs, swimming, and simple weights. I work out five days a week.

What are some of your favorite things to do?

I live for friends and family. It really doesn't matter what we are doing so long as we are together. My best friend lives abroad so our time together is virtual — Face Timing for an hour-plus on Thursday mornings. My mom lives nearby so she comes over often. We laugh hard together. Grad school and genetic counseling have been kind to me in the communities they have provided. Coffee with colleagues is a highlight of most days. New things: getting outside — my cousin took me sailing, which is a new love! Eating gourmet cheeses and bingeing Netflix on occasion.

Hobbies?

Origami. Bothering my older brother. Doting on my nephew. Cooking. Making yogurt.

Tell me about writing a lay-person's guide to dating, and why?

I joked about writing a guide to dating with CF. I don't know that I really have enough expertise to guide anyone, but I do love to trade stories of the challenges of disclosing CF and its myriad issues in the context of dating and relationships. Some of the kindest and strangest things have happened to me in the course of online dating.

Do you have a funny CF story?

I went on a first date with an ENT surgeon. He did not yet know that I had CF. To warm him up for that disclosure, I explained that I had been hospitalized for flu. We then talked about how doctors search for the rare when the culprit is common, and I explained how that happened during the hospitalization (with respect to liver issues, but I did not get that detailed). He replied, "Yeah, I mean, it's not like you're 35 with cystic fibrosis." "But I am!!!!!" I blurted out, pointing a finger straight at him in a moment of shock and awe.

How do you cope physically and emotionally?

I cope by reaching out to friends and family and by working out. I find talk therapy helpful at times. Wellbutrin is vital to my disposition. During really rough times, bingeing on stand-up and Food Network is therapeutic and also motivates me through treatments.

Who or what inspires you?

Older people with CF. My friend Mary, who made it through an unplanned lung transplant. My family and friends whose support and love sustain me.

Are you pro or opposed to socialization of people with CF, and why?

People with CF navigate tons of risks every day. We should be educated to make our own choices with respect to socialization. Because I am a worrier and tend to feel guilty and blame myself when my health declines, I would worry too much about the social exposure to make it worthwhile. But I am interested in how people make these choices and have very much enjoyed interviewing people with CF about their CF friendships as a part of my doctoral work. Anyone with memories of CF camp or opinions about CF, socializing, and cross-infection should feel welcome to contact me as it would help my ongoing doctoral work. My contact information is rmueller@sas.upenn.edu or 215-260-5523.

Do you have a motto you live by?

Viktor E. Frankl: **Between stimulus and response there is a space**. In that **space** is our power to choose our **response**. In our **response** lies our growth and our freedom. ▲

Andrea Eisenman is 54 and has CF. She is a Director of USACFA and is both the Webmaster and Executive Editor of CF Roundtable. *Her contact information is on page 2. Jeanie Hanley is 57 and is a physician who has CF. She is a Director and the President of USACFA. Her contact information is on page 2. If you would like to be interviewed for "In The Spotlight," please contact either Andrea or Jeanie.*

Confessions of a CF Patient:

From Alcoholism And Depression Toward Emotional Healing

By Mark Anthony Tremblay

I know this is going to come as a shock to most of you, but for many years beginning in my early teens I drank regularly to forget I had cystic fibrosis (CF). So many of my friends had already died. No matter how closely my loved ones tried to draw me to them, there was always a seemingly impregnable barrier that prevented me from being intimate with them. To be honest, and I'm ashamed to admit it, I was angry that I had to run the CF race while everyone else got to sit on the sidelines in lawn chairs. I was jealous whenever I heard my brothers or friends laugh lightheartedly because, despite my best efforts, I could never seem to achieve the same tone and pitch of levity. But, most of all, I felt a profound sense of loneliness: I had no one with whom to share my innermost thoughts and feelings because, by the time I started drinking, all the CF patients I knew had died.

It actually was the night I attended the funeral of my last friend with CF that I started "treating" the emotional weight of CF with alcohol, and later drugs. Initially it worked wonders. In fact, at first, alcohol gave me the "wings to fly." When I was drunk, I wasn't bothered by my constant coughing. I fit in with everyone, and had an identity not as a kid with CF, but as the craziest person at the party. And when I'd get too drunk, I'd get in fights, which earned me the respect of peers because I was tough despite the fact that I was by far the smallest person in my class. However, as the saying goes, very quickly alcohol "took away the sky." I couldn't handle life without it, I hated being

MARK TREMBLAY

sober, and I actually became so depressed when sober that only drinking would pull me out of it.

But before I get ahead of myself, let me set the stage by saying I was born in 1969 and diagnosed in 1970. I currently have around 50% lung function, am working full time, and have a pretty rich, full life. However, when I was born, my mom was told the treatments for CF were minimally effective; hard for the patient as well as the care giver; and, even if you do everything right, you'll be lucky if your child lives to his ninth birthday. Note, among the few treatments available at the time were mist tents, manual percussive therapy (administered by my mom mostly), and digestive enzymes that had the unfortunate side effect of digesting the stomach lining. Due to my mom's extraordinary and courageous efforts, I made it to my teens but my friends in the clinic did not. Watching all those kids get sick as well as suffering through my own battle with CF left me sad, angry, and bitter, which led me to eventually adopt the motto of "better to burn up than fade away."

By age 18, I had already been revived once after overdosing on drugs; was in several car accidents; stabbed in a fight; and in many scrapes with the law, including several nights in jail. In short, I was well on my way to burning up before CF could even get a chance to make me fade away. Despite outpatient counseling and heavy legal consequences, I couldn't stay sober so I was referred to inpatient treatment shortly after my 18th birthday.

June 20, 1987, was the day on which I entered the Clearbrook Treatment Center and the last day in which I medicated my emotions with drugs or alcohol. In treatment, I learned to live one day at a time; manage my life, including the emotional and physical burden of CF, without picking up; and to share my life with my new Alcohol Anonymous family, my genetic family, and my friends. All this helped to erode the profound loneliness and isolation that I'd experienced all my life, which had made alcohol and drugs so appealing in the first place.

After graduating from rehab, I lived in a halfway house with other sober men and it was there I decided to begin college. Initially, I thought, like a lot of young people with CF at the time, that I didn't have much time before my career would come to an end, so I enrolled in college with the intent of earning a six-month certification in Alcoholism and Substance Abuse Counseling. However, increm-

entally, as my health held out, I made the decision to complete the school year and then another, and then another, until I ended up with two master's degrees, running an inpatient and outpatient drug and alcohol treatment center. For the last 20 years, after obtaining my Master's in Public Administration from Syracuse University, I have been working for the New York State Medicaid program. My other master's is in psychology.

I have spent nearly 32 of my 49 years on earth sober. Sobriety has taught me to live in the moment, as well as to consciously seek to deepen my relationship with my higher power, which in my case is God and Christ Jesus, and follow His will for my life. When younger, I had felt a heavy burden for being chosen to run the CF race and resentful of all those around me who got to go home after watching me race for a few minutes or hours. However, sobriety and later my Christian faith slowly led me to accept and ultimately embrace my role as a person with CF and even leverage my pain to help others suffering from alcohol, drugs, depression, anxiety, and even cystic fibrosis. Presently, when I'm not working, spending time with family and friends, exercising, reading or writing, I'm helping others face their demons as a leader in the Celebrate Recovery program at my church.

Although I have been sober for the majority of my life, I still struggle almost daily with depression because, at times, I feel like a burden to my loved ones, including my wife and family; guilt because I've outlived so many of my friends with CF who faced their diseases more courageously or lived life more boldly than I; and regret that so many times I acted out of fear rather than faith. For example, when I graduated with my Bachelor's in Psychology and Organic Chemistry, I had hoped to go to medical school, but I feared that the physical demands would make me get sicker faster, so I opted to get an advanced degree in psychology instead. However, looking back, I regret that decision and many others that I made because, for most of my life, I felt it was important to keep my sights low if I wanted to live long. So I never really reached for the stars. In essence, I spent way too much of my time in the hourglass, watching the sand slip beneath my feet instead of looking beyond the glass to the horizon ahead. In retrospect, I have no doubt that had I known that I'd still have 50% lung function at 50 years of age, I would have made different choices.

However, through more than 50 hospitalizations, nearly a hundred IV runs, divorce, business failures, and many regrets, I stayed sober and continue to more deeply embrace my life's purpose as a person with CF in a world where depression, anxiety, and substance disorders are all too prevalent. Living my life sober with CF through all the ups and downs has taught me that sometimes my own pain is the single most important tool I have to reach the most alienated and hopeless who suffer from so many of life's ills.

> "In treatment, I learned to live one day at a time; manage my life, including the emotional and physical burden of CF, without picking up."

Moreover, despite my own depression, I am slowly learning to reach a little higher for the heavens every day and encourage others, even those with CF and other chronic and terminal illnesses, to do the same. I spent most of my life believing I was dangling at the end of a very thin branch, but now we know that limb is much sturdier than was once thought and, in fact, the life expectancy for CF patients has extended beyond 40 and will continue to push even further out as the effects of the new modulator drugs are fully realized, and other breakthrough treatments make their way through their respective drug pipelines. The outlook for CF patients is so much brighter today than it was in the '70s, so obtaining advanced degrees, having children, and even grandchildren, and retiring on time after a full career are well within reach for patients today. However, despite this newfound optimism, I believe because of my own and others' experiences, that protocols for CF care need to better prioritize screening, identifying, intervening, and treating patients who are struggling unsuccessfully with the emotional burden of CF, as well as other issues that may manifest as depression, anxiety, and substance use disorders.

In closing, I hope this article, as well as others I look forward to writing in the future, will help the CF community realize that, while we are on the cusp of an armistice in our war with the physical progression of this disease, there is still so much work to be done to understand the emotional and psychological impact on those who have spent many years in pitched battle on the frontlines. ▲

Mark is 50 and has CF. He has 32 years sober. He and his wife, MaryGrace, live in Poestenkill, NY. They love to ride their Harleys, go road cycling, and do ministry at their church. Their favorite vacation spots are Cape Cod, MA, and The Outer Banks, NC.

The Reality Of Fighting For My Life

By Emily Trout

Growing up with cystic fibrosis (CF) wasn't particularly eventful for me. Both of my parents come from a medical background, so bringing medicine and treatments into my daily life was something we grasped fairly easily. I had my share of hospital stays and sporadic health flares but, for the most part, I was able to have a loving, exciting childhood. In high school, I played sports with everyone else. I went off to college, away from home, and most kids there didn't even know I had CF. I could get away with physically looking normal, and I clung to that and hoped that would never change.

I, like many CF patients, hit a downward spiral when I turned 20. I went from keeping up with all my friends in college, to actually being "sick." Of course, my whole life I was technically considered sick, but when I say "sick" now it means something completely different to me. When I looked in the mirror I saw an undernourished pale body and dark circles under my eyes, just noticeable if you could see past my steroids-swollen face. My once horse-like hair was now straggly and wispy. Between the visible port scars, g-tube, and barrel chest I had developed, there was no hiding from strangers that there was something different about me. I was sick, and I hated it.

First came the constant lung infections treated by IV antibiotics, then the rest all happened so fast. Suddenly I was learning how to give myself insulin, how to set up a feed through my g-tube overnight, and how to get my port accessed. Within a year, I was given the talk. My doctor let me know that evaluation for a transplant should begin sooner rather than later, but he worried about my chance at being accepted into the transplant program at a Boston hospital, near where I live. He explained that their programs are fairly small and stick to straightforward patients, which I am anything but. He recommended I look into Cleveland Clinic, and we laughed in his face thinking that no way in hell are we moving to another state for this transplant to happen.

Well, he was right, and when I received my rather unsympathetic calls from the Boston transplant programs letting me know my application was denied, my heart sunk. Just accepting this lifestyle of constant pain, hospitalizations, and treatments was not an option. I couldn't just stop here after fighting so hard. It was decided: we were off to Cleveland.

EMILY TROUT WITH HER DOG BELLA.

I knew transplant evaluation would be grueling and long. That being said, I was profoundly underprepared for what it actually entailed. The days started at 7 a.m. and ended at 5 p.m., with a 30-minute break in between — if we were lucky. I was poked and prodded more times than I could count, and I did so many breathing tests I didn't even know existed. I met with every specialist possible, and I constantly had to be putting my best foot forward, because I knew how badly I needed them to accept me. It didn't take long for me to be wowed by every doctor we met, and suddenly I knew exactly why this hospital was so prestigious. It quickly became my mindset that if I am getting a transplant, it is happening here — nowhere else.

It wasn't a fast process. In fact, it was an entire year from my first evaluation until I finally was told "you are officially listed." Up until this point, we had still been living in Boston and traveling back and forth as needed. Then it all became real. This was the city that would save my life. I was listed in October and, by the end of December, we were packing up to move to Cleveland, while also keeping our house in Massachusetts.

What was I supposed to do in Cleveland? I had no friends or extended family there. I had no knowledge of the city. I had my mom and my dad, who was traveling back and forth every few days for work in Boston. We had our two dogs. That was it. The first week seemed like a month. My anxiety was through the roof and, to be honest, I wasn't handling it very well. I felt like there should be some easy cure, a pill I could take to make it all better and shut out the reality of the situation. But there wasn't, and even though I learned that the hard way, I'm happy I did.

With CF, we are faced with an intense isolation that no young adult should have to experience. I have found myself focusing on my "support system" since finding out I was listed. The social worker asked me, "can you write a list of your support system for me to

keep on file?" It made me ask myself who my support system actually was. There were the easy ones to list: my mom, my dad, my sister, my brother-in-law. My mother is a selfless full-time caregiver to me, and my other family members all have a very strong presence in my life. I can list my best friends from college, and friends from growing up. But then I was left to realize, there is no relationship I can list.

Now, I'm not in the kind of situation where I am left daydreaming of a flawless boyfriend who will sweep me off my feet. I'm in the kind of situation where I no longer really know how to even approach a relationship. As I said earlier, when I look in the mirror I see "sick." Even more so, I feel sick, and what fun is that to date? I want to be the girl who can go on a hike for a date without dying of oxygen deprivation and coughing fits. I want to be the girl who can have a couple drinks and dance all night, and not constantly be wondering when is an acceptable time to leave so I can finally get to sleep. I want to be the girl in a bikini who radiates confidence, not the girl who keeps her clothes on so she can hide the scars and g-tube.

Then I realized something about the real me. I am still the genuine, empathetic, happy-go-lucky girl I have always been. Being sick cannot take that away from me. So, I am learning how to live in the end-stage of this disease, while acknowledging that I am a girl worthy of love and all the other emotions a 26-year-old should be going through. Of course, I am in a position that makes it a bit harder to find someone of interest for a relationship, but I won't look in the mirror anymore and see what I thought was a girl weakened and beaten down by this destructive disease. My scars show the pain I have overcome. My g-tube shows that I am fighting for a healthy weight that will make my transplant recovery easier. My hair, my paleness, my dark circles are all shallow problems that can simply be fixed. I still hate my puffy, prednisone face, but I remind myself that it won't always look that way.

So, while I am still waiting on the list for transplant, it came down to one awareness that I know I need to carry with me to stay resilient. I own my emotions, I own my mindset, and I create my happiness. I have always loved writing, so I started online classes that further my education, while giving me a feeling of purpose. I connected with an old friend from home, who now has become a very close friend, and she always gives me an outlet to vent about my feelings and how to overcome them. I went shopping for beauty products, which may seem vain, but they made me feel better about myself and that's what matters. I started a blog to update loved ones about my health, which doubled in being a surprisingly useful way for me to express how I truly was dealing with life on the waiting list. I started wearing outfits that showed my port and my scars on my chest, and it felt good mentally to wear something that made me feel good physically. As for a relationship, I still haven't tackled that yet. That's okay though, because first I needed to remember that I'm deserving of a special relationship just like anyone else. Don't get me wrong, this is a process. I still haven't mastered this mindset. But I am going in the right direction, and learning to love my flaws. I'm starting to feel pride and strength when I catch people looking at my scars, because really all they are seeing is that something hurt me and I overcame it.

It may not sound like much, but it is all I have right now as I live this life of constant uncertainty. When you are waiting for transplant, all you want to do is live some wild dream life where you can check off all your bucket list items and never regret a single day. That isn't reality. Waiting for transplant means the hospital becomes your second home; you have more bad days than good; and you feel the toll this is all taking on your body. One thing I will never do is pretend that this journey is filled with joy and enlightenment. I don't want you to think that I have everything figured out, because I really, seriously, do not. All I can share of what I have learned so far through this process is that if you need help, reach out and ask. If you have the opportunity, find CF patients who have gone through transplant to talk to, because they actually get it. If you are self-conscious about how this disease has affected your appearance, remember that you look this way because you are a fighter. More than anything else, remind yourself daily that these hardships are just part of the battle you fought for a chance at a new life, and it was so worth it. ▲

"I am learning how to live in the end-stage of this disease, while acknowledging that I am a girl worthy of love and all the other emotions a 26-year-old should be going through."

Emily is 26 and has CF. She is from Boston, MA, and currently lives in Cleveland, OH, while awaiting her transplant.

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molecule for protein production) — and regulates bacterial motility, biofilm formation, and other virulent (pathogen-linked harmful) factors.

RpoN is also linked to antibiotic resistance. Researchers developed a so-called "molecular roadblock" in the form of a peptide (RpoN*), which can block DNA transcription controlled by RpoN and other factors.

Although previous studies showed that RpoN* expression reduced the virulence of laboratory strains of P. aeruginosa, its effect is not known in clinical isolates of the bacteria. So the researchers evaluated the effect of RpoN* expression on 12 different P. aeruginosa strains isolated from patients with CF, especially regarding virulence and antibiotic susceptibility. Four laboratory bacterial strains were used as controls. The features of the bacterial strains were either tested in culture plates (in vitro), or using live models (in vivo). Results indicated that RpoN* reduced the virulence caused by P. aeruginosa isolates derived from CF patients and suggested that RpoN* expression reduced the pathogenic effect of the bacteria in vivo. RpoN* also increased the bacterial sensitivity to cefotaxime, cefepime, ceftazidime, piperacillin, and imipenem.

https://tinyurl.com/y5pw4gl2

Lung Enzyme Cathepsin S Is New Therapeutic Target For CF And COPD

Blocking the enzyme cathepsin S can alleviate symptoms and reduce lung damage in cystic fibrosis (CF). High levels of cathepsin S have been reported previously in the lungs of CF patients, and they have been linked to increased inflammation and lung tissue scarring. In the first study, researchers used a CF mouse model and tested a cathepsin S blocking agent, called VBY-999. Results showed that animals treated with VBY-999 had a significant reduction in lung inflammation and damage, as well as in mucus obstruction. In contrast, experimentally inducing high levels of cathepsin S in a healthy mouse triggered increased mucus production, inflammation, and damage to the lungs, the team noted. Cathepsin S performs its activity with the help of a receptor protein called PAR-2. The team showed that in CF mice, blocking PAR-2 also alleviated lung disease symptoms, confirming a vital role for cathepsin S in lung damage.

https://tinyurl.com/y5bdolyu

P. aeruginosa Variants Contribute To Local Lung Inflammation In CF

Specific Pseudomonas aeruginosa bacteria called mucoid variants, which are linked to poor prognosis of patients with cystic fibrosis (CF), are associated with significantly greater regional lung inflammation. When P. aeruginosa first colonize the lungs of CF patients, they show a non-mucoid colony appearance in lab cultures, but as the disease progresses the bacteria often mutate into mucoid variants that form thick, stringy, and wet colonies. Mucoid variants tend to be more resistant to the effects of antibiotics and to be more difficult to be cleared by the immune system. Scientists also have found that mucoid and non-mucoid variants can collaborate to become more resistant to elimination mechanisms. CF commonly induces more damage to the upper pulmonary lobes. Researchers decided to investigate whether preferential upper lobe localization of certain P. aeruginosa variants could contribute to this. They found that P. aeruginosa mucoid and non-mucoid variants were distributed similarly throughout the CF lung lobes. In other words, they could not detect preferential upper lobe localization of specific bacterial variants. In addition, the upper and lower lobes of the CF lung did not exhibit significant differences in the amount of pro-inflammatory proteins. Still, they found that infections caused by mucoid variants, whether alone or in mixed-variant populations, were associated with significantly higher levels of pro-inflammatory signaling proteins. Thus, P. aeruginosa mucoid colony variants seemed to be promoting inflammation independently of their localization within the lungs.

https://tinyurl.com/y5lkokkr

Poor Oxygen Conditions May Promote P. aeruginosa Infection Over Other Pathogen In CF, Study Suggests

Poor oxygen conditions may help Pseudomonas aeruginosa bacteria outcompete other infectious agents, namely Staphylococcus aureus, a study suggests. Research suggests that P. aeruginosa produces specific molecules that target S. aureus, reducing bacteria numbers. Nonetheless, according to the researchers, studies estimate that a third of adult CF patients are infected with both types of bacteria, and this co-infection is associated with lower pulmonary function and more lung complications. Researchers investigated how oxygen availability — which is known to affect bacteria energy production — affects bacteria interactions in CF. Under normal oxygen concentrations, P. aeruginosa was indeed able to outcompete S. aureus. However, when bacteria were growing without oxygen — a condition called anoxia — P. aeruginosa no longer dominated S. aureus making it evident that anoxia facilitates S. aureus–P. aeruginosa co-existence. This study suggests changes in oxygen availability within regions of the CF lung is likely to influence interspecies interactions and in turn, potentially influence disease progression.

https://tinyurl.com/y38hzcgv

Common Virus Linked To Faster Disease Progression In Cystic Fibrosis

A new study has found that cystic fibrosis (CF) patients who have a common virus may experience faster disease progression than patients who do not have the virus. Signs of faster CF disease

progression included earlier times to lung transplant referral and reaching the final stages of the disease. The study looked at the cytomegalovirus, a typically harmless type of herpes virus that is often contracted during late adolescence and early adulthood. The researchers say their findings suggest the virus may be an unrecognized contributor to CF, but more research is needed to confirm whether the virus causes the disease to progress more quickly.
https://tinyurl.com/y6gmw27k

CF-associated Bacteria And Fungi Affect Each Other's Growth, Study Says

Physical and signaling interactions between two pathogen groups — Pseudomonas aeruginosa bacteria and Scedosporium fungi — result in a growth effect (inhibition or enhancement) of these microorganisms. CF therapies such as antibiotics or corticosteroids also affected the growth of the fungi. Antibiotic therapy may upset the polymicrobial (many types of microorganisms) environment, and consequently allow other pathogens such as fungi to establish. To better understand the cause of fungal infections in CF, the researchers evaluated the direct and indirect effects of P. aeruginosa as well as commonly used CF-related therapies on fungal growth. Results showed that physical contact inhibited both bacterial strains (about 60% inhibition) and fungal strains (100% inhibition). Regarding the use of corticosteroids and antibiotics, results showed that Scedosporium growth rates were in general unaffected by hydrocortisone treatment. Co-incubation with the corticosteroids prednisone and methylprednisolone resulted in reduced growth in the majority of the tested strains. Researchers then tested tobramycin, ceftazidime, and flucloxacillin. While the presence of tobramycin showed a dose-dependent effect on the growth rate of the strains, ceftazidime and flucloxacillin had highly vari-

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BOOMER ESIASON FOUNDATION'S PROGRAMS

CYSTIC FIBROSIS SCHOLARSHIPS

The Boomer Esiason Foundation Scholarship Program provides assistance to students with cystic fibrosis based on a number of criteria including: academics, athletics, arts, and more...
www.befscholarhships.com

LUNG TRANSPLANT GRANT PROGRAM

The Boomer Esiason Foundation Lung Transplant Grant Program provides grants to cover expenses including, but not limited to: temporary housing, food, and transportation costs.
www.befgrants.com

CYSTIC FIBROSIS PATIENT DISASTER RELIEF PROGRAM

In light of recent natural disasters, The Boomer Esiason Foundation created a fund that directly assists those affected families in the cystic fibrosis community.
www.esiason.org

TEAM BOOMER FIGHTING CYSTIC FIBROSIS

Team Boomer is a program that encourages people with cystic fibrosis to incorporate exercise into their everyday lives; provides an avenue for individual athletes in a variety of sports to raise money for cystic fibrosis; and offers assistance to grassroots athletic events looking for a cause to support.
www.teamboomer.org

BREATHE IN with GUNNAR ESIASON and SALTY CYSTERS
A CYSTIC FIBROSIS PODCAST

A series of audio and video podcasts, featuring Gunnar Esiason and the Salty Cysters, that highlight people with CF and the challenges they face.
www.gunnaresiason.com

JERRYCAHILL'S CYSTIC FIBROSIS PODCAST

A series of audio and video podcasts in which Jerry Cahill interviews people with CF who are living, breathing, and succeeding through the power of exercise, nutrition, and compliancy.
www.jerrycahill.com

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able results, leading to both higher and lower growth rates.
https://tinyurl.com/y5wj48en

Virus Can Help Treat Pseudomonas aeruginosa In CF, Study Suggests

A new study shows that bacteriophages, viruses that naturally infect bacteria, can be used to fight Pseudomonas aeruginosa. In a recent study, researchers described a cocktail of bacteriophages that was effective against Pseudomonas aeruginosa in two animal models of acute infection. The results showed that phage therapy is able to decrease lethality, bacterial burden, and the pro-inflammatory response caused by [Pseudomonas aeruginosa] infection. The data also suggested that phage therapy and antibiotic administration appears as a promising therapeutic approach, especially in order to reduce antibiotic doses and treatment duration.
https://tinyurl.com/yxhlhnah
AND
https://tinyurl.com/y5xg4xc4
AND
https://tinyurl.com/y5yh9xjm
AND
https://tinyurl.com/yxg2pcsm

Drug Resistance In Cystic Fibrosis Lung Infections Associated With Pseudomonas Bacteriophages

Bacteria involved in infecting the lungs of cystic fibrosis (CF) patients may get a boost from filamentous viruses found within them. The researchers used DNA sequence or quantitative PCR data to profile bacteria in lung or sputum samples, identifying filamentous bacteriophages (Pf phages) that were overrepresented in a significant proportion of older CF patients and patients carrying Pseudomonas aeruginosa bacteria that are drug resistant. The team suspects that the phages help Pseudomonas bugs in CF patient lungs, perhaps by boosting biofilm production and making it more likely that Pseudomonas will establish chronic infections in the lungs.
https://tinyurl.com/y5ozr7pv
AND
https://tinyurl.com/y5qplqx2

Developing A First-in-class Regenerative Gene Therapy For Cystic Fibrosis

The innovative OmniSpirant platform is based on the use of engineered stem cell exosomes as regenerative carriers for inhaled gene therapy. Exosomes are nanosized vesicles that are naturally produced by virtually all cells and are involved in cell-to-cell communication. Large volumes of recent research highlight the vast potential of stem cell exosomes as transformative regenerative gene therapy and medicines.

OmniSpirant's patent-pending method of surface engineering exosomes enables the exosomes to efficiently penetrate mucus and enter into the targeted lung cells, for the first time demonstrating widespread and efficient entry into the high number of cells that are required for truly successful CF gene therapy. The enhanced delivery also has the potential to maximize the regenerative effects mediated by the stem cell exosomes.

OmniSpirant is developing inhaled bioengineered exosome therapeutics, delivered by a tailored aerosol delivery method based on vibrating mesh nebulizer technology. The lead product, OSL001, is designed to combat the respiratory dysfunction caused by CF, a genetic disorder. The stem cell exosomes are therapeutic (regenerative, anti-inflammatory, antimicrobial and antifibrotic), non-immunogenic, and can also be tailored via genetic modification of the parent stem cells to create ideal inhaled gene therapy vectors for any lung disease. Additionally, it is expected that the exosomes will not cause genotoxicity as with mRNA gene therapy there is no risk of potentially carcinogenic genomic integration that is a major safety concern for integrating viral vector gene therapies.
https://tinyurl.com/yyg3hyu5

OSU Researcher Earns $3.3 Million Grant To Create Universal Cystic Fibrosis Treatment

A pharmaceutical sciences researcher at Oregon State University has received a five-year, $3.3 million grant from the National Institutes of Health to develop a way for cystic fibrosis (CF) patients to get molecular treatment via an inhaler, the university announced in a news release. The new treatment would be designed to work on every CF patient, regardless of his or her specific genetic mutation. The project is based around lipid nanoparticles carrying messenger RNA that can correct the underlying genetic defect that causes the disease: a mutation in the gene that encodes an ion transporter protein.
https://tinyurl.com/y2o9xgd9
AND
https://tinyurl.com/y2e2j6m8
AND
https://tinyurl.com/y5o6o347

PAAG Experimental Molecule Can Disrupt CF-related B. cepacia Biofilms

An experimental large sugar molecule called poly (acetyl, arginyl) glucosamine, or PAAG, was able to disrupt treatment-resistant biofilms formed by Burkholderia cepacia complex bacteria extracted from patients with cystic fibrosis (CF). Researchers decided to test a new experimental treatment strategy based on using PAAG, a complex large sugar molecule belonging to a class known as glycopolymers. Effects of PAAG were also found to be dependent on the specific isolates tested, establishing the most robust reduction in B. cenocepacia out of all the tested isolates. Treatment with PAAG also induced a reduction of biofilm thickness by approximately 75–80% on all tested bacterial species compared with those treated with inactive or control compounds. As a complement to the

biofilm mass measurement, the researchers also assessed the number of remaining live bacteria after biofilm disruption with PAAG treatment. Again, they saw a significant and dose-dependent reduction in live bacteria one hour after treatment, while treatment with inactive controls resulted in no significant changes.
https://tinyurl.com/yxp3x9qa

Vertex Selects Its Triple For Cystic Fibrosis

VX-445 (elexacaftor) is the compound that Vertex will add to its already marketed drugs tezacaftor and ivacaftor, after reviewing data from two clinical trials, to try to increase the number of CF patients eligible for treatment with one of its drugs. Vertex has been testing multiple triple regimens in an attempt to extend the proportion that can be treated with a Vertex drug to around 90% of people with the genetic disease. The company says it plans to file for U.S. approval in the third quarter of the year, and European approval in the fourth quarter. The decision means that Vertex has effectively stopped development of VX-659, its other frontrunner for the triple therapy. Vertex says it decided that the VX-445 triple combination regimen could benefit the greatest number of CF patients based on multiple factors, including favorable profiles for safety, tolerability and drug-drug interactions, the ability for co-administration with hormonal contraceptives, and the lack of photosensitivity.
https://tinyurl.com/yxfslvwa

CFF Awards TB Alliance $5.1M to Develop Therapies For NTM Infections

The not-for-profit organization TB Alliance will receive up to $5.1 million from the Cystic Fibrosis Foundation (CFF) to advance the discovery and development of treatments for multi-drug-resistant nontuberculous mycobacteria (NTM) infections in people with cystic fibrosis (CF). TB Alliance will partner with Johns Hopkins University over the next three years to find and test potential treatments for infections caused by the two NTM types: Mycobacterium abscessus and Mycobacterium avium complex (MAC). The group of bacteria that make up the different types of NTM, including M. abscessus and MAC, are related to those that cause tuberculosis (TB). In developing therapies for that disease, which are now being evaluated in patients, TB Alliance identified three classes of chemical compounds with the potential to target the two NTM bacteria. TB Alliance will individually screen these compounds, with a goal of producing therapies for CF that are more effective than the available treatments.
https://tinyurl.com/y4njz7p9

Phase 2a Trial Will Test Molgradex In CF Patients With NTM Infections

Savara Pharmaceuticals has launched a Phase 2a clinical study to evaluate the effectiveness of Molgradex in cystic fibrosis (CF) patients with chronic nontuberculous mycobacterial (NTM) lung infections. Molgradex is an inhaled form of artificially produced human protein granulocyte-macrophage colony-stimulating factor (GM-CSF), which normally is produced and secreted by several types of immune cells to trigger a response against pathogens. The open-label Phase 2a study, ENCORE (NCT03597347), will test the effectiveness of Molgradex in about 30 CF patients with chronic pulmonary NTM infection — caused by either Mycobacterium avium complex (MAC) or Mycobacterium abscessus. The study will include participants who are either taking an antimycobacterial regimen, are intolerant or non-responsive to NTM antibiotics, or failed to fulfill the criteria to start antibiotic treatment. The study's primary goal is three negative NTM cultures collected consecu-

Continued on page 40

Richard DeNagel – July 5, 1968 To May 10, 2019

USACFA mourns the death of Richard (Rich) DeNagel, age 50, from New York City. Rich died from complications that developed a month after his bilateral lung transplant. Rich was an avid writer for CF community blogs, and had been a columnist for *CF Roundtable.* He served on the USACFA board of directors from 2007 to 2010. Rich worked as a high school teacher, on and off, in California and New York, as his health permitted. Rich struggled with numerous health challenges with grace and courage. He was humorous, independent, driven, open, creative, and loved his friends and his life. He was preceded in death by his older sister to CF and is survived by his sister, Ann DeNagel.

TILLMAN continued from page 39

tively (with a four-week interval between them) from the sputum of participants. Secondary goals include other microbiological indicators, as well as lung function measurements, and patient-reported outcomes. The company also is conducting a Phase 3 trial (NCT03181932; AVAIL) testing another product called AeroVanc (inhaled vancomycin), to treat persistent methicillin-resistant Staphylococcus aureus (MRSA) lung infections in CF patients.
https://tinyurl.com/y3hcoq4j

SNSP113 (Previously SYGN113) Is An Inhaled Therapy That Aims To Ease Pulmonary Infections, Airway Congestion, And Inflammation.

SNSP113 contains an active ingredient called poly N (acetyl, arginyl) glucosamine, which is a type of complex sugar molecule. The treatment is reported to have a multi-faceted mechanism of action that might protect pulmonary function in CF patients:

Targeting and breaking apart bacterial biofilms. These are communities of bacteria that are attached to the airway epithelium and are usually resistant to antibiotics. By breaking the biofilms, SNSP113 could reduce antibiotic resistance to common disease pathogens, such as Burkholderia cepacia complex, Pseudomonas aeruginosa, and methicillin-resistant Staphylococcus aureus (MRSA).

Normalizing the mucus viscosity of CF patients, and to improve mucus transport and airway clearance by interacting with the mucin polymers, which are the major components of mucus.

Interacting with and disrupting the cell walls of the invading bacteria, to increase their permeability and allow greater uptake of antibiotics given to kill bacteria.

Reducing the inflammatory cascade of neutrophils, immune cells whose activity causes pulmonary tissue damage and fibrosis.

Lab data also suggests SNSP113 has strong anti-inflammatory effects at low doses. A single-ascending dose Phase 1 study (NCT03309358) tested the safety and tolerability at day eight of treatment with inhaled SNSP113 in 32 healthy people. This trial initially was a two-part study in healthy people and CF patients, but was stopped after what is reported to be the "successful completion" of its first part.
https://tinyurl.com/y22pgsq6

Trial Of Nebulized CF Candidate Completes Single-ascending Dosing

The RESTORE-CF clinical trial investigating Translate Bio's candidate therapy MRT5005 has completed the single-ascending dose regimen in patients with cystic fibrosis (CF). The ongoing Phase 1/2 trial (NCT03375047) is testing the safety and efficacy of single and multiple escalating doses of nebulized MRT5005, compared with each other and a placebo. MRT5005 is a messenger RNA product designed to deliver the correct sequence of the CFTR gene to lung cells so that they can produce a fully functional CFTR protein. MRT5005 is the first potential mRNA approach to specifically target the lungs.
https://tinyurl.com/y4d3v8b3

Potential CF Therapy Gets Boost After Contrafect Granted Nearly $7M

ContraFect will receive up to $6.94 million in funding from CARB-X to support the development of their proprietary therapeutic peptides — amurins — against antibiotic-resistant bacterial infections caused by gram-negative ESKAPE pathogens. The company intends to develop these compounds as potential therapies for pulmonary exacerbations of cystic fibrosis (CF) and hospital-acquired bacterial pneumonia. CARB-X (standing for Combating Antibiotic Resistant Bacteria Biopharmaceutical Accelerator) is an

organization that accelerates the discovery and development of alternate antibacterial therapies to curb the growing problem of drug-resistant bacteria. Amurins are bacteria-lysing agents, or lysins, naturally produced by viruses that kill bacteria (called bacteriophages). ContraFect's lysin platform uses bioinformatics and genomics to identify and produce amurins that can target a wide variety of bacteria.

In addition to free-floating bacteria, amurins work on bacteria that have attached to surfaces and formed biofilms.

https://tinyurl.com/y34sn825

AmorChem, UdeS To Advance CF Research Of Antibiotic Tomatidine

AmorChem has entered an agreement with Université de Sherbrooke (UdeS) and TransferTech Sherbrooke to optimize the antibiotic tomatidine for treating infections associated with cystic fibrosis (CF). Tomatidine is a naturally occurring product that can be extracted from tomatoes. Previous findings have shown that tomatidine and analogous (comparable) molecules have antibiotic properties, and may be useful to treat infections linked to CF. Research teams have identified the molecular target that tomatidine and related molecules act upon.

Preliminary findings suggest that tomatidine activity could be extended to treat a broad spectrum of bacterial infections, including Gram-negative and Gram-positive, two types of bacteria distinguished by structural differences in their cell walls.

https://tinyurl.com/y5c3jlev

Positive Preclinical Data On Potential CF Therapy ELX-02 Presented

Eloxx Pharmaceuticals presented new positive preclinical data for ELX-02, demonstrating that the therapy increases the levels of CFTR protein and its correct placement at the cell's surface in patient-derived organoids. ELX-02 is an experimental therapy to treat CF caused by nonsense mutations, in which a premature stop signal is inserted in the CFTR gene. This signal causes the cellular machinery to stop making the CFTR protein midway, forming an incomplete version of the protein that is rapidly degraded by the cell. ELX-02 is a eukaryotic ribosomal selective glycoside (ERSG) that binds to ribosomes, complexes that serve as factories to produce proteins inside cells. The therapy is designed to increase ribosome's skipping over nonsense mutations, and enable the production of sufficient amounts of full-length CFTR protein and lessen CF burden.

https://tinyurl.com/y4yf6j7l

AND

https://tinyurl.com/yyfqv5p2

Motif Bio Signs Agreement With Lamellar Biomedical

Motif Bio plc, a clinical-stage biopharmaceutical company specializing in developing novel antibiotics, announced that the Company has signed an agreement with Lamellar Biomedical Limited (Lamellar) under which Motif Bio will conduct an in vivo pre-clinical study evaluating iclaprim in combination with Lamellar's patented LAMELLASOME™ technology. Iclaprim has been granted U.S. orphan drug designation for Staphylococcus aureus pneumonia in patients with cystic fibrosis (CF). Lamellar's LAMELLASOME™ candidate LMS-611, which has mucokinetic (mucus clearing) properties, has demonstrated antibiotic potentiation (the enhancement of certain properties of antibiotics) and has European orphan drug designation for CF. The companies believe that, based on pre-clinical data with the two individual components, the combination could be a promising potential treatment for lung infections in patients with CF.

https://tinyurl.com/y3gffegq

ABBV-2222 Partly Corrects CFTR Function In CF Patients, Phase 2 Data Show

Treatment with ABBV-2222, an investigational CFTR corrector formerly known as GLPG2222, was well-tolerated and partially corrected the function of the CFTR protein, both alone and in combination with Kalydeco (ivacaftor), in a group of patients with cystic fibrosis (CF), Phase 2 clinical trials show. ABBV-2222 is a CFTR corrector that helps abnormal CFTR protein fold correctly, so it won't be destroyed by cellular agents and will be safely transported to the cell membrane, resulting in an increase in chloride transport. In both studies, patients carrying either one (ALBATROSS) or two (FLAMINGO) copies of the F508 deletion took ABBV-2222 orally once a day for 29 days. Patients participating in the ALBATROSS trial were also carriers of another gating mutation in CFTR, and were being treated with Kalydeco. The trials' primary outcome was to assess the treatment's tolerability and safety. Secondary goals included assessing the pharmacokinetic properties of the compound, meaning how the therapy is absorbed, distributed, metabolized, and eliminated from the body, as well as the effects of ABBV-2222 on sweat chloride, patients' lung function, and respiratory symptoms. Results showed that treatment with ABBV-2222 led to a significant reduction in patients' sweat chloride concentrations However, no significant improvements were found in patients' lung function, nor in their respiratory symptoms. Treatment-related adverse events were reported in 29.2% of patients in FLAMINGO and 40% in ALBATROSS. Most were mild to moderate in severity, and included respiratory, gastrointestinal, and infection complications. No deaths or therapy discontinuation associated with treatment adverse

Continued on page 42

events were reported in any of the trials.
https://tinyurl.com/y4pwrjcz

Potential CF Therapy PTI-428 Receives Orphan Drug Status In Europe

Proteostasis Therapeutics received orphan drug designation from the European Commission for its investigational CFTR amplifier PTI-428 in development for the treatment of cystic fibrosis (CF). This recognition of the therapeutic potential of PTI-428 in Europe follows previously granted orphan drug, breakthrough therapy, and fast track designations from the U.S. Food and Drug Administration. PTI-428 is being developed to increase the amount of cystic fibrosis transmembrane conductance regulator (CFTR) — the protein that is defective in CF patients. Proteostasis Therapeutics is developing PTI-428 as part of its proprietary triple combination regimen that also includes PTI-808 (a CFTR potentiator), and PTI-801 (a third-generation CFTR corrector). Recent results from a Phase 1 trial (NCT03500263) demonstrated that treatment with the triple combo for 14 days could effectively improve lung function, with a 5% increase in mean absolute ppFEV$_1$, and reduce sweat chloride levels.
https://tinyurl.com/y28j4qpl

FDA Panel Narrowly Backs Approval Of Inhaled Mannitol For Adult Cystic Fibrosis

This is the second time this therapy has been discussed during an FDA advisory panel. In 2013, the Pulmonary-Allergy Drugs Advisory Committee unanimously recommended against approval of inhaled mannitol. At the time, panel members noted that the two phase 3 clinical trials discussed at the meeting were not adequate to determine efficacy and raised safety concerns about increased risk for hemoptysis. The current advisory panel meeting focused on a third trial. The primary endpoint was forced expiratory volume in 1 second (FEV$_1$) over 6 months. Important secondary endpoints were pulmonary exacerbations and Cystic Fibrosis Questionnaire-Revised scores. Only adults were included in this trial due to concern about hemoptysis in the pediatric population. Although the trial met its primary endpoint by showing a statistically significant increase in FEV$_1$ with inhaled mannitol, the improvement was modest. Furthermore, the studies did not show a benefit with inhaled mannitol with respect to reduction in pulmonary exacerbations. The dry powder formulation of mannitol is an inhaled hyperosmotic agent designed to increase mucus clearance. The treatment has two components: the drug product, which is a spray-dried mannitol powder in capsules and a dry powder inhaler. The proposed dose is 400 mg of mannitol, administered through oral inhalation of ten 40-mg hard gelatin capsules twice daily using the inhaler. Each inhaler is used for 1 week and is then discarded. Although the FDA is not required to follow the recommendations of the advisory committees, it usually does.
https://tinyurl.com/y66mw4nc

Manuka Honey To Kill Drug-resistant Bacteria Found In Cystic Fibrosis Infections

Researchers have found that using Manuka honey could offer an antibiotic alternative to treat antimicrobial resistant respiratory infections in cystic fibrosis (CF) patients. Using lung tissue from pigs, experts treated grown bacterial infections mimicking those seen in CF patients with Manuka honey. The results showed that it was effective in killing antimicrobial resistant bacteria by 39% compared to 29% for antibiotics, while improving the activity of some antibiotics that were unable to function effectively by themselves. Honey and antibiotics combined killed 90% of the bacteria tested.
https://tinyurl.com/yxa3xk8x

CF Patients With Lung Allergy From Fungi Respond Well To Xolair, Study Says

Xolair (omalizumab), a medicine used for allergic asthma and chronic hives, is effective for treating lung allergic reactions caused by fungi in patients with cystic fibrosis (CF). Xolair is a biologic agent (an antibody) approved for the treatment of uncontrolled allergic asthma and chronic spontaneous urticaria (chronic hives of unknown cause). It is given as an injection under the skin. There is evidence that Xolair may also benefit patients with ABPA, given its ability to block immunoglobulin E (IgE), the major type of antibody involved in allergies. However, the team emphasized the lack of randomized clinical trials to confirm Xolair's benefits assessing both clinical and laboratory outcomes, including steroid requirement, ABPA exacerbations, and improvement in lung function. Also, more data is needed to define the optimal dose and duration of treatment with Xolair before this expensive therapy can be recommended as a routine treatment approach.
https://tinyurl.com/y62co66h

Nitric Oxide Reduced Burden Of Mycobacterium Abscessus Lung Infections In CF

Inhalation of nitric oxide as an add-on therapy may help reduce the burden of serious bacterial lung infections, namely Mycobacterium abscessus. Nitric oxide (NO) is a naturally occurring gas that has been shown to help the lungs resist pathogens by triggering the immune system. In fact, when applied directly to the airways, it can attract immune cells and has the potential to clear bacterial, fungal, and viral infections. In a previous study, researchers showed that inhaled NO at 160 parts per million (ppm) for 30 min, five times a day for up to 26 days, reduced the M.

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abscessus bacterial load and improved lung function in two CF patients. Now, researchers evaluated the safety and effectiveness of inhaled NO in nine CF patients with hard-to-treat M. abscessus lung infection. The study's main goal was to assess safety. Additional outcomes evaluated lung function and exercise tolerance, using the forced expiratory volume in one second and 6-minute walk test (6MWT), respectively. Additionally, researchers evaluated the load of M. abscessus in the lung. Results showed that at the end of the treatment, patients' lung function and exercise capacity had increased compared to the start of the trial. The inhaled NO also reduced the bacterial load of M. abscessus infection. https://tinyurl.com/y674eusg ▲

Laura is 71 and has CF. She is former director and President of USACFA. She and her husband, Lew, live in Northville, MI.

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- You can reach **USACFA** and ***CF Roundtable*** at any time by e-mail at **cfroundtable@usacfa.org**
- Send your questions of a general nature regarding legal issues that relate to CF to our legal advisor: **Beth Sufian, Esq., 712 Main, Suite 2130, Houston, Texas 77005. E-mail: CFLegal@sufianpassamano.com**
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