CF Roundtable®
A PUBLICATION FOR ADULTS WHO HAVE CYSTIC FIBROSIS
AUTUMN 2023

Navigating The Final 10%: A Hopeful Journey In Cystic Fibrosis

By Chandra Ghose, Ph.D., CSO, Emily's Entourage

Introduction

Cystic fibrosis (CF) has long been a relentless adversary, affecting thousands of lives worldwide. As the research community advances along the path of clinical treatments, a specific group remains in the shadows, the final 10% of people with CF (PwCF) with rare and nonsense mutations, facing the most formidable challenges.

The Initial 90%: A Journey of Advancement

The past has seen remarkable advancements in CF treatment, resulting from the dedication and perseverance of scientists, clinicians, PwCF and their families. The first major milestone was the discovery of the CFTR gene in 1989, which paved the way for a deeper understanding of the genetic basis of CF. Since then, various therapies have been developed, aiming to address specific CFTR gene mutations and restore proper cellular functioning.

CHANDRA GHOSE

The introduction of CFTR modulators has been a game-changer for PwCF with specific CFTR mutations. These drugs have shown promising results in improving lung function, reducing hospitalizations, and enhancing overall well-being. These successes in CF research have fueled optimism and underscored the importance of continued investigation of modulators for rarer mutations such that the label for these modulators may be expanded to include additional PwCF.

Global access to affordable CFTR modulators is a major challenge faced by PwCF. Access may vary depending on several factors, including the country's healthcare system, regulatory approvals, insurance coverage, and individual patient circumstances. Studies have shown early treatment with CFTR modulators can have a positive impact in lowering the burden of disease on PwCF. (https://www.cysticfibrosisjournal.com/article/S1569-1993(22)00083-2/fulltext) While CFTR modulators are standard of care in high-income countries, PwCF in low- and middle-income

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EDITOR'S NOTES

This issue we're focusing on the 10% left behind in our community: those who aren't eligible for any of the modulators and those who can't tolerate the side effects. For the focus topic, **Laura Mentch** shares her detailed Trikafta journal entries focusing on the side effects she experienced. **Andrea Eisenman** writes about her nonsense mutations and her ongoing struggle with her lung functions. Additionally, the co-founder of Emily's Entourage, **Emily Kramer-Golinkoff**, shares her insight as part of the 10%. **Camille Richards** talks about her late diagnosis and finally receiving the correct treatment has worked wonders for her health despite not being eligible for modulators.

We're deeply saddened to share the passing of one of our long-time columnists and speaker, Isabel Stenzel Byrnes. You can read about her life and legacy on p.34.

In their "Pearls of Wisdom" column, **Dr. Xan Nowakowski** notes the importance of embracing life by reckoning intentionally with death as someone included in the 10% left behind. **Beth Sufian**, in part 1 of her "Ask The Attorney" column answers questions about social security benefit overpayments. For our Pet's Perspective column this issue, **Colleen Adamson**, on behalf of Penny, regales readers with the joys of being in the top 10% of cutest dogs and her perspective in watching her fur mom take Trikafta. **Maggie Williamson** shares her recipe for Vegan Sweet Potato Chili in her "Culinary Corner" column this issue. **Aimee Lecointre** takes up the helm as our research guru with her first "Research Roundup" column. In the second installment of "Chaptered Lives," **Andrew Corcoran** writes about his travels to Madrid and what it means to truly live, not just survive. **Katie Lockwood** shares the importance of advocating for yourself as a parent with CF in her new column "Salty Parenting." This issue we're highlighting **Jacob Greene** for our "In The Spotlight" section—Jacob is our newest board member, a prior scholarship winner, and a full-time student earning his MD.

In other community news, thanks to the generous support of the Helen M. Eisenman fund, BreatheStrong+ has partnered with Beam to provide free adult Beam CF on-demand classes through the end of September 2024.

Congratulations to our recent higher education and arts scholarship winners! USACFA now offers three scholarships! You can read about each of our scholarships on p.25 or, head to our website where you'll also find the application, requirements, and deadline for each. Additionally, we're always looking for people to interview for our In The Spotlight column, new writers and columnists, as well as new directors. We love hearing from our readers! Reach out to us if you're interested in sharing your story in an upcoming issue. You can also find a list of focus topics both on our website and on p. 3 of this issue. We can connect you with our newest volunteer, **Melissa Teeman**, if you need help sharing or writing your story.

In the words of Effie Trinket from *Hunger Games*, may the odds be ever in your favor, Sydna.

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Research Roundup

Compiled by Aimee Lecointre

Eloxx Pharmaceuticals Announces Final Data Assessment From Phase 2 Combination Clinical Trial Of ELX-02 In Class 1 Cystic Fibrosis Patients

Eloxx Pharmaceuticals, Inc. has announced the final data assessment from the Phase 2 clinical trial of ELX-02 in combination with ivacaftor in Class 1 CF patients with at least one nonsense mutation. The final data assessment includes a reanalysis using change in ppFEV1 from Day 1 instead of baseline, as multiple patients experienced disease progression between screening and treatment. Initial results from this trial were reported in September 2022. The Phase 2 combination clinical trial of ELX-02 was designed to evaluate safety and assess biological activity in G542X nonsense mutation Class 1 CF patients as monotherapy and in combination with ivacaftor after 5 weeks of treatment. Summarized final analysis follows: 6 of 13 patients entered trial from monotherapy arm and had a decrease in lung function due to disease progression. Treatment with ELX-02 stabilized disease overall and resulted in a clinically relevant increase in ppFEV1 in six of thirteen patients based on change in ppFEV1 at the end of treatment compared to day one. Patients with higher baseline sweat chloride levels had increased responses to treatment as indicated by sweat chloride concentration. ELX-02 was generally well tolerated in the trial, with no treatment-related serious adverse events noted.

https://tinyurl.com/ytrx86ed

AIMEE LECOINTRE

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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, high-resolution photo of yourself as well as your name and contact information. Email all submissions to: articles@usacfa.org. Or go to our website: www.cfroundtable.com/publication.

Autumn (November) 2023: The 10% Left Behind (Current issue)

Winter (February) 2024: Organ Transplants (Deadline December 15, 2023) Have you had a transplant? If so, for how long and can you tell us about your journey? Tell us the good, the bad, and the ugly. What can you share to offer hope to those who are waiting? Were you prepared for all of it? What do you wish you knew before embarking on this endeavor? Are you waiting for one or deciding whether to get one? What are your hopes or fears for receiving a transplant? Are you in need of a second organ transplant? If so, why? What issues (e.g., diabetes, high blood pressure, anxiety, etc.) have you encountered after receiving a transplant?

Spring (May) 2024: CF and Cancer (Deadline March 15, 2024) Have you been diagnosed with cancer, either pre-transplant or after? If so, what kind? Has CF made it more complicated or challenging to treat? Have you had recurrent skin cancers post-transplant? What coping strategies have you used to process being diagnosed with cancer in addition to having CF? Were you angry, resigned, or sad and how do you deal with those emotions? What advice would you give others who are fighting cancer?

Summer (August) 2024: CF and Travel (Deadline June 15, 2024)

ASK THE ATTORNEY

Social Security Benefit Overpayments: Part I

By Beth Sufian, J.D.

In the past three months, we have received many questions from *CF Roundtable* readers about Social Security benefit overpayments. In 2023, Social Security has been looking at records and finding thousands of overpayments. A person receiving Supplemental Security Income (SSI) or Social Security Disability Insurance (SSDI) could receive an overpayment notice for payments made many years ago that were not correct payments. A person can also have an overpayment if they are receiving a Social Security benefit based on a parent who is deceased, disabled, or receiving Social Security retirement benefits.

The information provided in this article is only meant to be general information. This article is an overview of the Social Security Administration (SSA) rules regarding overpayments but is not a comprehensive discussion of every issue that may arise with an overpayment.

Nothing in this article is meant to be a guarantee that a person will not have an overpayment if they take certain actions in terms of assets or work income or work activity.

The information provided in this article is only meant to be general information. Nothing in this article is meant to be a guarantee that a person will be eligible for SSDI, SSI, or any other government program.

Question 1:

What is an overpayment?

Answer:

An overpayment is simply a payment from the SSA to a person who receives Social Security benefits over the amount the person is entitled according to SSA rules. The amount of the overpayment is the difference between the amount the person received and the amount the person is due if all Social Security rules are applied.

When the SSA determines that a beneficiary has received an overpayment, it will send a notice of overpayment to the person (or to the person's representative payee). This Notice of Overpayment is sent on paper by U.S. Mail to the person or their representative's address on record with the SSA. The Notice of overpayment should include:

1. The amount of the overpayment;
2. The reason for the overpayment; and
3. Repayment options.

Usually, a notice of overpayment will describe how the SSA calculated the overpayment. The description of how the overpayment was calculated is important in understanding whether the amount of overpayment claimed can be successfully challenged. Also, the events that caused the overpayment will have occurred before the beneficiary receives the notice of overpayment and the SSA may not realize an overpayment occurred for several months or years.

BETH SUFIAN

Usually, the notice of overpayment will include information on how to appeal an overpayment or request a waiver of overpayment. However, some notices of overpayment make a demand for repayment without instructions on how to appeal or request a waiver. A person can contact the SSA regarding filing an appeal or requesting a waiver of the overpayment.

Question 2:

What causes overpayments?

Answer:

The causes of overpayments are different for SSI and SSDI beneficiaries because the continuing benefit eligibility rules are different for each program.

Question 3:

What are the common causes for overpayments for SSI beneficiaries?

Answer:

The three most common causes for overpayment are changes in household income, changes in household assets, and a change in marital status.

A person is eligible for SSI if the person meets both the medical and the financial criteria, as well as other program requirements. The medical criteria are listed in the SSA regulations. A person's symptoms must meet the listing of medical symptoms or be as severe as the listing **and** have daily limitations that prevent an adult from working more than a certain amount each week or a

child from engaging in the normal activities of daily living. SSI requires that the individual with the disability live in a household with income and assets below the SSA limits.

A common cause of an SSI overpayment is an increase of household income. It is important to note that the SSA counts household income—not only the income of the person with the disability but also a spouse's income. Additionally, whether the income is earned or unearned also makes a difference in how income is counted by the SSA. Income that is earned—such as wages and salary—are counted by the SSA and will be applied to reduce the SSI monthly benefit due. Every $2 of earned income will reduce an SSI benefit by $1. Unearned income is also counted by the SSA, and every $1 of unearned income will reduce the SSI monthly benefit by $1. There are SSI programs that may allow the ability to save work income under a PASS plan or another SSA work-related program.

Another common cause of an overpayment is having household assets that exceed the limit of allowable resources. The SSA often refers to assets as available resources. The SSA's household asset limit for SSI is $2,000 for a single adult or one adult and one child. An adult couple or two parents living in the same household with a child who receives SSI have a $3,000 asset limit at all times. For a person receiving SSI, the SSA does not count the value of a home the person owns and does not count the value of one motor vehicle.

Changes in marital status can also affect eligibility for SSI because it affects the size of a household and usually affects household income and assets. These effects on eligibility may occur even if the health, income, or assets of the individual beneficiary are unchanged. This is because the SSA considers the household's income and assets when making SSI eligibility determinations. If the household includes a new spouse who has income or assets over the applicable limits, then a beneficiary living in the household will become ineligible because the beneficiary no longer lives in a household with income and assets below the applicable limits. The SSA regards two people as married if they are living in the same household and are married under the laws of the state where they live. The SSA also regards two people as married if they are holding themselves out as a married couple to the community in which they live.

There are other causes of overpayments for SSI beneficiaries, but these three are the most common for SSI beneficiaries.

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MILESTONES

Please share the milestones in your life with our readers. Your successes and achievements may serve as a source of motivation for others in need of an infusion of "positive mental attitude" in the pursuit of their goals. Send us a note specifying your "milestone." Include your name, age, address and phone number. Mail to: **CF Roundtable, 9450 SW Gemini Drive, PMB43881, Beaverton, OR 97008-7105.** Or email to: **cfroundtable@usacfa.org**

ANNIVERSARIES

Birthday

Mike Darrar
Post Falls, ID
57 years on August 25, 2023

Beth Sufian
Houston, TX
58 years on August 13, 2023

Wedding

Colleen and Scott Adamson
Alexandria, VA
26 years on June 28, 2023

Mike and Kathy Darrar
Post Falls, ID
33 years on August 11, 2023

Andrea Eisenman and Steve Downey
New York, NY
15 years on September 13, 2023

Dr. Xan Nowakowski and Dr. J. Sumerau
Lakeland, FL
7 years on June 25, 2023

Transplant

Colleen Adamson
Alexandria, VA
Bilateral lung transplant
25 years on July 3, 2023

PET'S PERSPECTIVE

Being Part Of The 10%

By* Penny, *translated by Colleen Adamson

Hi, everyone! Penny here. I was very excited to hear that the focus topic of this issue is the 10 percenters, because I am one of them! I assume this group is also known as the Top 10% Cutest Dogs Ever!

Not to brag, but I will: I am one of the founding members of this group. But you knew that, right? You've seen my picture! We 10 percenters do not really do anything except what comes naturally—being cute! We do make this world a better place with our very presence. It is not easy to make this world better right now; it is pretty messed up, or so I hear and feel. Is it me or is it getting hotter on my walks around the neighborhood? The grass is really dry, too; it hurts my paws a little bit to walk on it.

Have I mentioned my favorite color is pink? Studies have shown that pink is a very soothing color, and I must agree. Plus, it adds to my cuteness. I have new pink argyle socks that I wear inside so I don't slip on our hardwood floors. I love them. My old socks were black and gray—boring! I also have pink jackets and leashes and, of course, my collars have all been pink. I also have pink poop bags, because I have a neighbor dog who is a boy and didn't want pink poop bags, so he gave them to me. He does not know what he is missing!

I think I am still cute despite being very old (16.5 years old). Cuteness is really more of an attitude than a physical trait. I still have an attitude! I may not walk as quickly or see and hear as well, but I look like I still got it. That's half the battle right there. As long as you look good, you can kind of feel good. Fake it 'til you make it, am I right? Momma jokes about this with her golf game. She looks like she knows what she is doing because she has the right clothes and clubs, but when she goes to hit her (pink) ball, that can be another story. Sometimes she hits well, other times not so much. Same thing with CF; Momma likes to look good even if she is not feeling well, because it helps her feel like a human again.

LEFT: PENNY WITH COLLEEN ADAMSON IN THEIR DRIVEWAY. ABOVE: PENNY DRESSED AS A BEE. "CAN I BE ANY CUTER?"

Momma has been feeling really good since she started taking Trikafta in 2020. At first, she had swelling in her ankles, and was worried that was a sign of kidney issues with her transplanted kidney. Once that was ruled out, Trikafta became the prime suspect. Edema was not listed as a side effect of Trikafta, but that was the only thing that had changed with her medicine routine. Her CF doctors took her off Trikafta temporarily, to see if the edema went away, and it did. Her doctors then put her back on it, but at a lower dose of just the two morning pills on Mondays and Thursdays. That did the trick and no swelling has happened since she has been on the lower dose. Plus, it is still keeping her sinuses clear on this lower dose!

Well, I think I have covered everything about this top 10 percenter stuff, aka Top 10% Cutest Dogs Ever. If you think you are part of this esteemed group, please consider writing for Pet's Perspective. We would love to hear from you and, more importantly, see how cute you are!

To my fellow 10 percenters: stay positive, stay well, and of course, stay cute!

Colleen Adamson is 54 and has CF. She and Scott got Penny when she was 10 weeks old in 2007. Penny passed away at the end of August. She was the love of their lives and they miss her very much.

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Question 4:

What are the common causes for overpayments for SSDI beneficiaries?

Answer:

Common causes of overpayments for SSDI beneficiaries include when the amount of earned income or the amount of work activity exceed the allowable limits for SSDI.

The causes of overpayments for SSDI beneficiaries are different for SSI beneficiaries because SSDI is a different benefit program with different rules for eligibility. Among the many differences between SSI and SSDI is that SSDI eligibility is based on the person's contributions to the Social Security system through payroll withholding. SSDI benefits are intended for workers who are no longer capable of substantial gainful activity because of an illness or injury.

To be eligible for SSDI disability benefits, a person must be unable to engage in substantial gainful activity. A person who is earning more than the SSA's allowable monthly amount can be found to be engaging in substantial gainful activity and therefore ineligible for SSDI. The monthly allowable amount of income is updated each year by the SSA.

In 2023, the maximum amount of work earnings a person receiving SSDI benefits can receive each month is $1,470 per month in work income before taxes are taken out of the work earnings. The Social Security Act specifies a higher amount of substantial gainful activity for statutorily blind individuals. For a blind person, the current allowable amount is $2,460 per month for 2023.

The SSA's amount of monthly income considered to be substantial gainful activity ordinarily excludes any impairment-related work expenses, which will be addressed below. A SSDI beneficiary who earns more than $1,470 a month (or $2,460 a month if blind) may become ineligible for further benefits. Social Security may not become aware of the earnings that make a person ineligible until sometime after the earnings are received and the person became ineligible. Payments made to a beneficiary that were not supposed to be paid will result in an overpayment.

Earnings is not the only measure of substantial gainful activity. The number of hours each week a person engages in substantial work activity is also limited. Working 20 or more hours a week is ordinarily considered substantial gainful activity by the SSA, even if the wages earned are less than the allowable monthly income. Social Security may not become aware of a person's substantial work activity until months or years have passed. Any payments made to a beneficiary that were not due will result in an SSDI overpayment. Overpayments may arise in ways other than those described in this column.

The next issue of *CF Roundtable* will include a column that discusses options a person has once they receive an overpayment notice from the SSA.

If you have questions about laws related to Social Security benefits, Medicaid, Medicare, health insurance, employment and education rights, you can contact the CF Legal Information Hotline at CFLegal@sufianpassamano.com or 1-800-622-0385 to set up a time to speak to an attorney. All calls are confidential and there is no cost to the caller. The CF Legal Information Hotline (CFLIH) is generously funded by the CF Foundation, but CFLIH employees are not employed by the CF Foundation. The CFLIH is now in its 25th year. ▲

Beth Sufian is 58 years old and has CF. She is an attorney who focuses her law practice on disability law and is the 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.

LECOINTRE continued from page 3

Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Regulates The Production Of Osteoprotegerin And Prostaglandin E2 In Human Bone.

Bone loss is an important clinical issue in patients with CF. Whether or not the CFTR plays a role in bone cell function is not yet known. In this study, evidence was provided that inhibition of CFTR-Cl(-) channel function results in a significant decrease of osteoprotegerin (OPG) secretion accompanied with an increase of prostaglandin (PG) E(2) secretion of primary human osteoblast cultures (n=5). The data suggests that in bone cells of CF patients, the loss of CFTR activity may result in an increased inflammation-driven bone resorption (through both the reduced OPG and increased PGE(2) production), and thus might contribute to the early bone loss reported in young children with CF.

https://tinyurl.com/4r5r8tcr

Clinical Outcomes At 9-10 Years Of Age In Children Born With Cystic Fibrosis Transmembrane Conductance Regulator Related Metabolic Syndrome

There is limited data on cystic fibrosis (CF) transmembrane conductance regulator-related metabolic syndrome (CRMS) outcomes beyond infancy. The goal of this study was to analyze outcomes of infants with CRMS up to the age of 9-10 years using the CF Foundation Patient Registry. From 2010-2020, there were 8,765 children with diagnosis of CF or CRMS entered into the CFFPR with sufficient diagnostic data for

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PEARLS OF WISDOM

Memento Mori: Lessons From The 10% On Making Space For Death

By Xan Nowakowski, Ph.D., M.P.H.

The past few months have been a bit strange in my world. I've mourned over two dozen different people close to me and my family since late April. For the first two weeks of June, I did not go more than 36 hours without news of another person dear to me passing away or entering hospice. Beyond the obvious difficulty of experiencing so many bereavements right on top of one another, I also felt disoriented and overwhelmed by the specific nature of these losses. With my feet underneath me a bit more firmly now, I'm finding important lessons beneath the surface of my grief.

Most of us in the adult CF community have become quite familiar with the heartbreak of losing our age peers to the disease. But only three of these individuals I've grieved recently had CF. I've come to understand that my frame of reference differs quite substantially for coping with the loss of people from within the CF community versus those outside it. In the process, I've developed a deeper appreciation for how my own experiences as a member of "The 10%" of CF patients ineligible for CFTR protein modulators shape my approach to understanding and dealing with death on a broader level.

The initial FDA approval of Trikafta for adult CF patients in October 2019 brought entirely justified excitement to our community. Despite being ineligible for the drug myself due to extremely rare CFTR mutations, I felt enthusiastic about the possibilities the new triple combo therapy could open up for many other adults living with CF. At the same time, I felt skeptical for a number of reasons that look quite wise indeed as we approach the four-year anniversary of that milestone.

I can't say I felt a lot of "fear of missing out" over Trikafta itself, given the drug was already well known to stress the kidneys. Living with CF-related chronic kidney disease, I share my care clinical providers' sense that such a drug would only be worthwhile for me if my lungs declined badly. But there's certainly something to be said for having choices in one's care—which as usual remain unevenly distributed in our population. The adult CF community mirrors broader healthcare access demographics for the United States. I've told you that familiar story many times before in these columns.

> *"I never had to deal with that feeling of having my supposed salvation snatched away—because I never expected to be saved at all."*

XAN NOWAKOWSKI

I've also been heartened by progress—among both CF organizations and the providers who care for us—on amplifying stories and experiences from marginalized patients within our community. It's been a while since I've talked with a provider who thought a Black person couldn't have CF, for example. And it's also been a while since I've talked with a provider who questioned the importance of considering the intersections of CF with gender and sexuality. So we have much to look forward to as a community in our care horizons and our partnerships with clinicians.

We're also still dying from CF. And in a world where many of our peers do have access to modulators and are thriving on those therapies, sometimes being a 10%er can feel a bit like being the Ghost of Christmas Future.

Most children were probably not as taken with the classic film adaptation of *A Christmas Carol* as I was. How very neurodivergent of me to have such a niche hyperfixation! I watched that movie over and over, my favorite part of all being where the skeletal apparition of Christmases yet to come points Alastair Sim's Scrooge to his own headstone at the cemetery. If my parents

found any of this strange, they certainly didn't let it ruin my fun.

My mother even helped me dress up as the Ghost of Christmas Future for Halloween in sixth grade; we made me a robe that perfectly matched the design of the one in the movie. I wore that thing so much it started falling apart. Perhaps we could have picked a sturdier material—but then again, does something not lasting forever mean it wasn't worth having in the first place?

As my spouse likes to say when I introduce a key point obliquely: *I knew you were going to land the plane.*

Call me an optimist or just a weirdo who liked dressing up in morbid costumes a bit too much, but I don't mind thinking about my own mortality. Not presently while I enjoy a period of reasonably good health with my lungs and nutritional health, and not in my 20s when my lungs were steadily declining and I was terribly malnourished from untreated exocrine pancreatic insufficiency.

Maybe I also liked the Ghost of Christmas Future because it made being skinny look artful and poignant. I very much enjoy feeling more spry than I did in those difficult days a decade ago, having color in my cheeks and not shedding hair everywhere, but I'm not exactly playing with a full deck in the flesh department. It sure beats having to live on medical nutrition supplements and having no ability to digest most solid foods, though. So I don't mind still doing a decent impression of an enduring favorite character.

The Ghost of Christmas Future didn't talk, though. It just sort of pointed at things and left Scrooge to draw his own conclusions. Clearly my impression needs some work yet. I'm also well aware of the irony of referencing *A Christmas Carol* as a foundation of my own understanding of mortality, given a story about sad white Anglo-Saxon folk in Victorian England might not be the best frame of reference for a less-sad multiethnic person in the present-day U.S. But we never do see the Ghost's face in that film adaptation. I like to think of it as an Everyghost of sorts, reminding us all that we'd do well to make the most of the time we have on Earth.

I also find it kind of comforting that life doesn't last forever in this regard. Because what if I *don't* always succeed in making the most of my time? Living with that other neurodivergent oddity of eidetic memory means that when I feel poorly about a choice I made or the outcome of my efforts, those experiences stay with me in perpetuity with the same vivid detail they had at the outset. When I was admitted to intensive care at 23 with a very poor initial prognosis for survival, I thought about that as I weighed what to say to my clinicians and my loved ones. I thought about the temptation of letting go, of unburdening myself at last of "the chain I forged in life" as Charles Dickens put it. It certainly had its merits as an idea.

> *"The question of how and when we'll die also seems far less important in my view than the question of how we'll live in the meantime."*

Yet I realized very quickly that I didn't feel ready to go. I didn't feel like I'd completed the mission in life—because I'd never gotten the opportunity to use everything I'd experienced up to that point in fighting for a conclusive diagnosis and appropriate healthcare to change the landscape for others following after. I was okay with dying; I wasn't in the best situation at the time and I was suffering on more fronts than just the physical. But I wasn't okay with not getting the chance to make a difference.

Walking out of that hospital under my own power—or at least walking across the parking lot given staff had to take me to the exit in a wheelchair for liability reasons—didn't seem like riding off into the sunset of an immortal future. I wouldn't even get my CFTR mutations identified for another 10 years after nearly losing my life that spring. Rather, it felt like getting started on a long and arduous journey that would bring rewards as it also challenged me in unprecedented ways. I think I read the writing on the headstone quite clearly then; I've kept walking towards it steadily ever since.

Sadly the past couple years have brought not only many more deaths in our adult patient community, but also many wrenching stories about Trikafta and other modulator drugs causing more problems than they solved. It's no stretch to say that these drugs are miracles for the people who benefit strongly from them. It's likewise true that some people have wound up worse off for trying a drug that was supposed to ease their path enormously. I've seen both stories play out for friends and loved ones and felt glad to be on the sidelines of that journey. Living with a deadly progressive disease was never going to be easy. But I never had to deal with that feeling of having my supposed salvation snatched away—because I never expected to be saved at all.

In the social and behavioral sciences we talk about *cognitive dissonance*

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countries are left without lifesaving treatments even if they are eligible.

The Final 10%: Facing Unmet Needs

While the progress in CF research is commendable, for the final 10%, the journey has been fraught with obstacles, as their unique genetic profiles render them unresponsive to conventional treatments. This segment comprises individuals with nonsense and rare CFTR mutations for whom current therapies offer limited benefits. Furthermore, some PwCF who are eligible for modulators may develop side effects that prevent them from benefiting from such treatments.

Embracing Innovative Approaches

Gene therapy holds the potential to revolutionize the treatment landscape for the final 10% of CF patients. By addressing the root cause of the disease at the genetic level, this cutting-edge approach seeks to deliver functional copies of the CFTR gene to restore cellular function and thwart the debilitating symptoms of CF. These advances are now in clinical trials. (https://clinicaltrials.gov/study/NCT05668741). ADD, https://classic.clinicaltrials.gov/ct2/show/NCT05248230)

One promising avenue of research lies in gene-editing technologies. Gene-editing tools can provide new gene therapy strategies to achieve permanent correction such as Zinc Finger Nucleases (ZFNs) and transcription activator-like effector nucleases (TALEN), CRISPR gene editing (base and prime). While this approach is still in its infancy, preclinical studies have shown encouraging results, opening doors to exciting possibilities in the near future.

Editing approaches to correct *CFTR* include mutation-specific homology directed repair (HDR), ablation of splice mutations or insertion of a 'super exon' by HDR. Adenine base-editing (ABE) promotes efficient single base pair changes with minimal undesired byproducts, even in non-dividing cells. The ABE class of editors for A•T-to-G•C modifications offer certain advantages in the context of CFTR nonsense mutations.

Base editing offers a means to repair rare *CFTR* mutations such as W1282X. Solutions to limit or eliminate bystander effects, identifying the appropriate airway cell types for targeting and thus optimal delivery technologies, additional methods such as homology-independent targeted integration (HITI) without on-target indels or bystander effect seen with HDR and base editing, will be crucial for further development of DNA repair in the context of CF. (https://academic.oup.com/hmg/advance-article/doi/10.1093/hmg/ddad143/7256331?login=false)

> "Drug repurposing is a strategy to identify new indications for approved or investigational drugs that are outside the scope of the original medical indication."

Gene therapy is not without its challenges. The successful delivery of therapeutic genes to target cells remains a complex puzzle to solve. The lung is a prime target for genetic delivery. The greatest barrier to enabling clinical translation of genetic therapy for CF remains the lack of an effective delivery system of nucleic acids to the lungs. Due to the inherent nature of CF disease, complexities of lung clearance and innate immune defenses in the pulmonary milieu, *in vivo* delivery of genetic material to the target cells effectively and efficiently at functionally relevant levels is critical. The potential need for repeated dosing, the inability to effectively target the necessary cells in the airways for sustained effects, low efficiency due to the presence of host factors such as mucus secretions, inflammation and host immune responses, size restrictions on the genetic load, off-target safety and toxicity are all concerns that need to be addressed prior to any genetic therapy becoming a reality.

Viral vectors such as adeno-associated (AAV) viral and lentiviral platforms are making strides toward the goal of delivering genetic therapies to the lungs of CF patients that address some of the issues mentioned above. (https://spirovant.com/2022/11/05/poster-621/)

With rapid advances in material science and nanotechnology, non-viral methods of delivery of DNA and RNA to lung epithelial cells also hold great promise and avoid some of the safety concerns of using viral vector platforms. (https://recodetx.com/wp-content/uploads/2023/02/Ishimaru_GRC-2023.pdf)

Additionally, exploring combination therapies might offer a more comprehensive solution for individuals with complex CFTR mutations. By targeting multiple aspects of the disease simultaneously, these combinations could maximize therapeutic benefits and potentially revolutionize CF treatment for the most challenging cases. Drug repurposing of therapeutics could potentially provide novel therapeutic options for individuals with CF. Drug repurposing is a strategy to identify new indications for approved or investigational drugs that are outside the scope of the original medical indication. (https://openres.ersjournals.com/content/9/1/00495-2022)

Final Thoughts

The final 10% of CF patients embody the essence of courage and resil-

ience and encompasses individuals from diverse backgrounds, ethnicities, and geographic locations. Their journey represents the unfinished chapter in the narrative of CF research and treatment. As the research community perseveres in the pursuit of gene-based therapy for the final 10% of CF patients, increased funding, continued research, and advocacy are vital to transform hope into reality. Collaboration between academia, pharmaceutical companies, patient advocacy groups, and regulatory agencies such as the FDA and EMA is crucial to accelerate gene therapies tailored to the specific needs of the final 10%. ▲

Chandra Ghose serves as the Chief Scientific Officer of Emily's Entourage, a non-profit organization that funds research and drug development for individuals in the final 10% of the cystic fibrosis (CF) community who do not benefit from currently available therapies.

Before joining EE, Chandra founded Bioharmony Therapeutics, an early-stage biotech startup specializing in developing novel antimicrobials to combat drug-resistant bacterial infections.

Chandra holds a Ph.D. in microbiology from New York University School of Medicine and a bachelor's degree from Saint Louis University in biology and theology. Chandra has trained as a postdoctoral scientist at the Aaron Diamond AIDS Research Center, an affiliate of Rockefeller University and Harvard Medical School in the Division of Infectious Diseases, Massachusetts General Hospital.

Chandra was born in Kolkata, India. She now resides in New York City with her husband, daughter, and their pandemic puppy, Jaws. Chandra may be reached at chandra@emilysentourage.org.

> "One promising avenue of research lies in gene-editing technologies."

NOWAKOWSKI continued from page 9

as a central driver of human suffering. This is basically the idea that it feels weird when your expectations diverge substantially from your reality. When that divergence is negative, it feels *horribly* weird. So I do worry about framing a clinical intervention—whether a CFTR modulator drug or anything else—as deliverance from mortality. Even people in the adult CF community who live to be very old indeed will eventually die. When they do, CF will likely be what kills them. We all have to die somehow; living with a fatal progressive disease inevitably stacks the odds on what will finally take us out.

The question of how and when we'll die also seems far less important in my view than the question of how we'll live in the meantime. If the past few months have taught me anything, they've focused me firmly on the importance of embracing life by reckoning intentionally with death. And, moreover, the importance of having support in doing that reckoning—people to process with intensively and spaces to share openly. It has concerned me to see some CF organizations shifting away from talking frankly about death as part of the journey. That does more harm than good in my experience.

We can and should celebrate the journey of adult life with CF expanding to include many wonderful things for many wonderful years before the end of life comes. But as a 10%er who's never been much inclined toward waiting for a miracle, I've kept the specter of death centered in my rearview mirror with full knowledge that it will catch me one day. I'm not trying to outrun it but rather forging steadily ahead at my own pace.

Having support from my own friends, colleagues, and family in practicing that acceptance enriches my quality of life every day. Being part of USACFA gives me structured opportunities to both receive that support myself and spread it to others in the adult patient community. I'd like to see every organization serving people with CF nurture that culture in kind. Without those spaces for getting comfortable with our own mortality and that of those we love, the light suffusing our lives will dim and flicker. So I return anew to the lessons of *A Christmas Carol* in embracing how guided tours of darkness can ready us powerfully to embrace the light of the time we each have left. ▲

Dr. Alexandra "Xan" Nowakowski is 39 years old and has CF. Xan is a director of CF Roundtable, *in addition to being a medical sociologist and public health program evaluator. They currently serve as an Associate Professor in the Geriatrics and Behavioral Sciences and Social Medicine departments at Florida State University College of Medicine. They also founded the Write Where It Hurts project (www.writewhereithurts.net) on scholarship engaging lessons from lived experience of illness and trauma with their spouse, Dr. J Sumerau. You can find their contact information on page 2.*

CHAPTERED LIVES

Young Lungs, Old Soul

By Andrew B. Corcoran

Fifty percent in five years. In 2002, 50% of the people who received a bilateral lung transplant would be dead within five years. That statistic haunted me. Each day and every night, I would think of the time I had left and what, if anything, I could do with it. Weeks and months would pass by and my breathing after the transplant was unlike anything I had known. It was perfect. I could run, walk, hop, leap, sing, laugh, cry, and love and my body would be right there with me, allowing me to experience what I imagined everyone's body did. Yet, that idea of time winding down and the clock running out was still central to my daily existence. Would I be one of the 50%? Would I make it past the first year, or the second? If I did, what would that mean?

I knew what to do with a short lifespan—I had thought of not much else for years, but the idea of living 10 or 20 or even 30 years after the transplant was too much for my mind to handle. I had no idea what I would do with that amount of time. As those first few years passed, I experienced temporary psychotic episodes from severe anxiety. PTSD, psychosis, and survivor's guilt followed me around like a ghost, haunting my dreams. I would lie awake, staring at the ceiling and, beyond that, into the heavens, where my mind would escape into the insanity of a life clock ticking backwards.

This was the inevitable outcome of not dealing with the trauma that cystic fibrosis requires. 50% in five years. Yet, in early 2007, I was alive. Still breathing. Cystic fibrosis had changed me in ways I wish it hadn't. The mere fact that I was 19 and *needed* a transplant was difficult to reconcile. My thought process regarding the transplant was simple—someone had to die so that I could live. It was too much. It was too heartbreaking. And so I became angry. I was angry at the world. I was angry at God. I was angry with myself. I couldn't allow myself to live a life without overwhelming thoughts of sadness and death, and grief for the person who had passed. The gratitude I felt for that human being became all-encompassing. I felt like I needed to cure cancer or solve world hunger in order to pay back the gift I was given. I was alive, but I had only been *surviving* for so long that this new freedom of life and breath was foreign to me. Those first few years were like seeing technicolor in a black and white world and, rather than taking in all the wonders of nature and various, beatific shades of life, I closed my eyes and heart to the world.

> *"My thought process regarding the transplant was simple—someone had to die so that I could live."*

ANDREW CORCORAN

These were the feelings that led me to board a plane for Madrid in January of 2007. Moving to Europe seemed like an opportunity to finally realize what living life might mean. I spoke no Spanish. I knew no one in Madrid and I had no real idea what I would do there, but just the thought of beginning anew was enough to bring a smile to my face for the first time in years.

My first night there, I was supposed to meet an older man who had a room for me. I arrived at his apartment building and rang the doorbell. No one answered. It was snowing outside and the snowflakes were beginning to pile up on the ground. I was exhausted from the trip so I wrapped my bags around my limbs, securing them as tight as I could, laid down on the cold concrete, closed my eyes, and went to sleep. However, that idea of starting over, or more accurately, *starting* life, brought me peace.

At some point, in the early hours of the morning, I felt a light kick to my shoulder. Looking up, a man stood above me, pointing down, saying one word, "Americano?" "Yes," I replied.

I began teaching English at various magazine publishing houses, trying to make ends meet, writing when I could, cooking dinner for one every night, and strolling along the bustling streets of Madrid. I met some people at an English school who quickly became close friends. They would see me take medicine and I'd tell them about cystic fibrosis and the

transplant, but the conversation ended there. Eventually, the thoughts of my disease relocated from the front of my mind to the back, becoming a distant memory replaced with laughter and smiles. I would check in with my transplant team back in Philadelphia from time to time, but the truth was that my body and lungs were so healthy, I was able to largely forget about the prior five years, not exactly running away from myself, but not dwelling on it either.

On a random March evening on the streets of Madrid, I wandered into a local bar and, within minutes, my whole life changed.

Back in 2000, before my transplant, I completed a barrage of tests. Physical examinations, walking tests, breathing tests, weeks of bloodwork, X-rays, MRIs, CT scans, cardiac catheterizations, as well as emotional and mental health exams. I met with psychiatrists and social workers, all asking me similar questions. *Why did I want to live? Why did I want the transplant? What would I do with my life if I were lucky enough to get the transplant?*

My answer was always the same. I wanted to live so I could fall in love. Just once I wanted to know that feeling. I was only 18 years old and love seemed like a good enough reason to want to live.

That March night in 2007, at that bar in Madrid, was the night I finally realized why I had gotten the transplant in the first place. The woman behind the bar was named Carol. Carol was Brazilian and I fell in love the moment I saw her. We closed the bar and sat and talked for hours, but cystic fibrosis and my transplant never came up. I remember it was raining outside. We stood under a streetlamp in the middle of the night without a soul around, silent, crisp raindrops falling around us. We stood there together and, just like that, in that very moment, every ounce of anger, every fragment of sadness or anxiety or trauma or despair melted away like a snowflake on wet concrete.

Four months later, in July, I received an email from my childhood doctor, Dr. Daniel Schidlow. He had run the pediatric cystic fibrosis center at St. Christopher's Hospital for Children in North Philadelphia. To my surprise, Dr. Schidlow happened to be in Madrid and was being honored at the Spanish Foundation for CF. He invited me as his guest. The gala took place at a large banquet hall in the center of Madrid. Thousands of people were in attendance and Dr. Schidlow was the guest of honor. He was being presented an award for his work by Spain's Foundation of Cystic Fibrosis. For his speech, he introduced a slideshow—a case study of a young man with CF who had become very sick and nearly died. In the end, the young man had received a bilateral lung transplant and was alive and well. Just then, on the slideshow, my own picture popped on the screen and a spotlight shone down on me in the center of the room. Unbeknownst to me, the case study was my own. I was the young man. I sat there in shock as a thousand people rose to their feet and applauded. It was only then did I realize that the date was July 21. It was my five-year transplant anniversary.

> *"Moving to Europe seemed like an opportunity to finally realize what living life might mean."*

I got my transplant on July 21, 2002. I was 22 years old. Like most of us with cystic fibrosis, our childhood is different from that of other kids. If we're lucky, that difference can be minimized, but, for many of us, at some point, that minimal interference grows into something larger. Something more sinister. I first truly got sick at 18. From 18 to 26, my life had essentially been put on hold. Life had hit the pause button and, although I intellectually understood how and why, my emotions were still mired in bewilderment and labyrinthian misunderstanding. What I didn't know before and what I eventually came to understand was that it was love that offered a respite from the disease. Love provided a necessary understanding of what a post-transplant life could be.

The individual fight against CF can be exhausting and challenging and wrought with tears and grief, but there is a power that comes with that as well. There is a knowing sense that life will wax and wane like ongoing tides, moving closer and further from the shore, mimicking the ways in which CF is closer and further from taking control of daily life. But, like the tides, there will always be a period of relief. CF will recede into the background and life once again becomes what we choose to make it. At 27, I had learned this lesson and, for the next six years, I chose a life of putting CF second and my own wishes and dreams first. Of course, like any patient with CF, there does come a time when cystic fibrosis demands attention, demands action, and, once more, becomes the centerpiece of life. In 2013, my own CF demanded exactly that from me. A demand that I could no longer ignore and a chapter of my life that would turn my life upside down one more time. ▲

Andrew Corcoran is 43 years old and has CF. He received a lung transplant in 2002. He now lives in South Jersey with his family and friends. He is a writer. Andrew's email is acorcoran@usacfa.org.

FOCUS TOPIC

THE 10% LEFT BEHIND

What Nonsense!

By Andrea Eisenman

Once I had my lung transplant in 2000, I stopped going to a CF clinic. The transplant center advised me that they were now my main pulmonologists and medical providers. If I needed to see other specialists, they would recommend them to me at NY Presbyterian—a one-stop shopping experience. It was working great until my enzymes stopped aiding the digestion of food. At that time, I was taking Pancrease capsules. All of a sudden, about eight to ten years after transplant, my digestion deteriorated. And, due to that, I had stomach aches, diarrhea, and frequency of running to the bathroom. This all became untenable for an active lifestyle, so I asked the transplant center to help me with my enzymes. They recommended I see my old CF center with my pediatric pulmonologist. But my previous pulmonologist and his team had moved to the Children's Hospital at NY Presbyterian. I now had to see the adult CF care team at NY Presbyterian. I made an appointment; they recommended I start using Creon caps, and things vastly improved in my gut. During my follow-up visit with them, the CF doctor asked if had any other issues. I did: I had heard about the new modulators that had been developed and seemed quite successful for many people whom I knew and read about. I guess for some, taking these new drugs was transformational.

I asked the CF center if they could test for my mutations because, the last time I was tested via sweat test, there were only about 14 known mutations for CF at that point, which was possibly around the 1990s. I was not positive for any of them. So I embarked on finding out my mutations in the hope that, if my lungs started failing, I would have a back-up of taking these miracle medications. This time it was a saliva test for my CF markers, and they tested for over 2,000 known mutations. I was

ANDREA EISENMAN WITH HER DOG, TRIXIE.

then called about the results, which were puzzling to me. I was homozygous for a nonsense mutation called W1282X. It is common among Ashkenazi Jews.

I didn't know what my mutations meant at first. But I later found out that W1282X was a nonsense mutation and meant I could not benefit or even try the new Vertex drugs that were potentiators first and now modulators (Trikafta). I was a bit crushed emotionally. At that time, I was not experiencing anything major with my transplanted lungs. It was 16 years before I was put on IV antibiotics for an exacerbation. I was fortunate. That soon changed as IV antibiotics became more frequent, usually at least once or twice a year due to my recurrent sinus infections, which then infected my lungs. I had sinus surgery almost every three to four years to keep my sinuses clear. I then started hearing that those on corrector medications (at the time it was Kalydeco) were experiencing an improvement in their sinuses, even if they had lung transplants. I was truly jealous then. But I had to just keep doing what I had to do. I didn't obsess about being left out. I was still doing relatively well with a decent lung function in the 70s.

Then my health status changed abruptly in 2017 when I was diagnosed with a form of lymphoma called Post-Transplant Lymphoproliferative Disease (PTLD). I was angry at getting cancer after living what I considered a "healthy" lifestyle of exercise, mindfulness practices, and eating decently. And while the treatment for PTLD was not horrible, it did involve IV infusions of Rituxan that made my lungs feel inflamed for one to two days afterward and made me short of breath. This occurred initially when I was on weekly doses of the treatment. As my treatments were more spread out—monthly at first and eventually as little as once every three months—they were not so obviously affecting my breathing. Then, PTLD returned after two years of remission. This time I was in a clinical trial receiving T-cell therapy that took donor T-cells from those with the Epstein Barr virus (EBV), which is the catalyst for this type of lymphoma. These infusions made my body mount an attack on the EBV in my own cells.

After this therapy went on for

eight months, it did not rid me completely of lesions. But because they were localized in my right axilla, I was advised to complete a month-long treatment of radiation. This involved daily visits for 28 days receiving radiation directed at my armpit, and hence, I was warned that part of my right lung may be affected by inflammation and scar tissue. Thankfully I am still in remission for PTLD. But it has only been a year. My EBV is watched monthly for elevated numbers. A consistent rise in numbers could indicate that the lymphoma lesions have returned. I get a PET scan twice a year to get a definitive measurement that cancer is either still in remission or recurring again. After all of these treatments, I noticed my lung function was slowly sliding into the 50s, where it remains today. I also have more shortness of breath.

Due to my declining lung function, my doctor tried putting me on massive doses of steroids starting with 800 mg of solumedrol IV for three days, then tapering to 60 mg of oral prednisone. Every three days I tapered down 10 mgs to finally get back to my normal 5mg dose. The high doses of steroids didn't make a difference in my breathing; they made me crazy in my own skin and increased my blood sugars astronomically. My doctor said that there was one more treatment to try, but it involved a hospitalization and a bronchoscopy first to rule out infection, acute rejection, or fungus. Once my lungs were free of anything harmful, I would be admitted to the hospital for five days to receive antibodies to halt the progression of the decline of my lungs. But this treatment would also wipe my immune system, so I could not be around people or in areas with others I didn't know, like a movie theater or indoor venues (good bye, Broadway shows!). This was considered one of the only treatments left for me to take safely.

The antibodies were given at the end of May this year. They remain in the body for three months. I am now about three months post treatment. I am again feeling short of breath and know my options are limited. There is still hope for the remaining 10% who are unable to take current modulators—new inhaled options like gene therapy and mRNA particles inhaled into people's lungs to correct the gating process for chloride and sodium. But, unfortunately, those with solid organ transplants will not be able to join clinical trials due to the risk of inflammatory response, cancer, or possible rejection. If shown to be effective for those in the trials, however, I may be eligible sooner than expected if the results are demonstrated to be beneficial and safe. I am still excited that new treatments like these and gene editing may yet be treatments for us all to benefit from systemically, not just via inhalation. It seems researchers and CF care centers are working valiantly to find new ways to get our CFTR to function correctly, specifically for those in the remaining 10% population.

> "I then started hearing that those on corrector medications were experiencing an improvement in their sinuses, even if they had lung transplants. I was truly jealous then."

Even after receiving the antibodies, my lung function was not expected to drastically increase. Just not reduce further. So far, that is the case. I am holding at around 50% with some shortness of breath on exertion. If my lung function starts to slide further down, getting another lung transplant might be my only option to staying alive. This option may not be available to me due to having cancer (that is activated by having a suppressed immune system, which allows EBV to thrive). In order to be considered for another lung transplant at NY Presbyterian, I would have to be in remission for another two years, three total. Otherwise, I would have to search elsewhere and relocate to another center and city where I might be considered as a viable candidate with only one year in remission.

It can be frustrating to continue to feel left out of benefitting from all that is being done to cure 100% of the CF population. I do not obsess about this but rather go about my day to day while staying on top of my care and being openminded about new research as well as just continuing to enjoy life through my spouse, friendships, good food, sports, and my dogs. All of them feed my soul and keep me going so I can spend more quality time with them all. I remain hopeful and positive that, eventually, I may live to see CF cured. What a day that will be! ▲

Andrea Eisenman is 58 and has CF. She recently realized that her initials are AGED: Andrea Gail Eisenman Downey (her husband's surname)! She lives in New York, NY, with her husband Steve and dogs, Willie, Roscoe, and Trixie. Andrea is the Executive Editor for USACFA. She enjoys cooking new recipes, playing pickle ball, biking, tennis when possible, and staying active as her health allows. Her contact information is on page 2.

Late Diagnosis And In The 10%

By Camille Richards

I was diagnosed with cystic fibrosis (CF) at the age of 19. It came after one of my siblings did genetic testing in preparation for IVF. As a young child, I spent time in the hospital on three separate occasions for my lungs and even spent time on home oxygen when I was four. I frequently had a cough that felt embarrassing, especially for a shy girl on testing days at school.

I didn't know very much about CF when I went in for the sweat test and definitely had no clue what it would mean to have a diagnosis. After my sweat test results came back positive, I went to my first CF clinic appointment. I was expecting it to be a normal, quick doctor appointment and was ill prepared for five hours of talking to clinicians, doing tests, and learning about this disease. During that appointment, they collected blood to complete genetic testing, which later came back with two Class 1 mutations. I didn't think much about the results at the time.

Starting on CF meds was incredible. I remember after my first dose of pulmozyme, I was walking with friends to a campus event and I kept coughing up huge chunks of mucus. It was amazing the difference the breathing treatments made for me. It was both gross and remarkable to see how much would come out of my lungs during breathing treatments. The changes were drastic for me and these symptoms that had been my normal eased for me.

In 2019, I started a masters in social work program. It was my lifelong dream to become a therapist and I was thriving. At the beginning of 2020, I had a lung exacerbation that took me out of school and my internship for a couple weeks. On my second day back in school, my state shut down for COVID-19. Life changed drastically and suddenly CF took a front seat. I had my first pancreatitis attack later that year, resulting in a month-long stay in the hospital and four months with a nasojejunal tube because of persistent pain and inability to tolerate food. I first became involved with the CF community during this time.

CAMILLE RICHARDS

I got to learn about Trikafta and other modulators by hearing firsthand accounts as friends and acquaintances started it. I remember bringing it up to my doctor and her telling me about what Class 1 mutations meant and that it was highly unlikely any modulator would work on me due to my genetics. I have always felt like my CF is unique but typically it felt like mine was unique in terms of not being that scary disease other people have because of my late diagnosis. Also, the recent bout of pancreatitis felt like the bigger issue, whereas it seemed like others with CF struggled with their lungs more than anything. This was the first time that being unique was considered a bad thing.

For me, starting on CF-specific treatments made a huge difference for my lung health. I went from an almost constant cough to being able to sit through classes without coughing and I had a significant reduction in both bronchitis and pneumonia episodes. While they are not the same as modulators, the experience was similar to what I've heard others describe with the modulators. There are a lot of exciting studies being done currently and a community that I know is fighting for every single one of us. I don't think it will be long before more advances are made. This is a community that knows how to fight, advocate, support, and beat the odds. I've watched it happen before and I see the progress happening in real time. ▲

> "I remember my doctor telling me about what Class 1 mutations meant and that it was highly unlikely any modulator would work on me due to my genetics."

Camille Richards is 27 years old and has CF. She lives in Woodscross, UT. She is a practicing therapist specializing in helping individuals with OCD and anxiety-related disorders. In her free time, she enjoys art, cooking, and maintaining her role of favorite aunt to her 32 nieces and nephews.

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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

Notes From My Trikafta Journal

By Laura Mentch

Bending away from my concerns about the cost, I listened to the excitement in our community about the triple-combo modulator and Dr. Francis Collins's presentation at NACFC and asked about starting Trikafta. My F508Del mutation let me into the growing circle of those eligible for CFTR modulators. After a few short weeks, I had insurance approval, copay support, and a patient support specialist at Vertex. On December 2nd, the pharmacist and case manager from the specialty pharmacy called me to discuss dosing and potential side effects: dizziness, headache, diarrhea, and elevated liver enzymes. It was very fast coordination.

In the dark December days of 2019, I started Trikafta.

Preparing to share my experience with you, I'm reminded of the early, not-so-easy days with the magic pills.

☐ 1st week: *sputum!, runny nose, headache, nausea, sad, teary, agitated.*

Okay, I might be agitated from being told "you definitely need oxygen" while picking up results of overnight oximetry testing at the respiratory care company. As the nurse explained the results and consequences of low oxygen, I see another person's name and a different doctor at the top of the page.

☐ 2nd week: *headache, bloating, exhaustion/fatigue, hunger.*

☐ 3rd week: *body changes, gurgling, burping, sadness, dry eyes, my new pants don't fit, stool changes.*

☐ 4th week: *dry, scratchy eyes, disrupted sleep, extreme sadness.*

Deep into January I am sleepless and sad. Our nurse coordinator hears my discouragement, uncommon alongside the joyous response to Trikafta. We tried alternate dosing: taking the two orange morning (happy) pills in the evening and the blue evening (sleepy) pill in the morning. It didn't help.

"Our nurse coordinator hears my discouragement, uncommon alongside the joyous response to Trikafta."

LAURA MENTCH

My patient support specialist calls frequently and I continue to tell him about my side effects. Hoping my experience would get better, I opened a second box of Trikafta.

☐ 5th week: *sad, revved up in p.m., busy, another day–not so sad.*

☐ 6th week: *constipation, night sweats, no sleep :(.*

In February, I wondered how to tease out the mood of the cold, dark, snowy/icy winter with my sad mood.

Meanwhile, I had more testing for cardiac concerns with medication changes. The repeat overnight oximetry results were good, so I let go of the worries I carried about transitioning to supplemental oxygen.

☐ 7th week: *sleepy/tired, no sleep :(, bowel movements at 4a.m., feeling flu-like, still tired, sleepless after 1a.m., headache.*

☐ 8th week: *no sleep, visual aura without headache.*

☐ 9th week: *sad, TIRED, sleepless, revved up at bedtime, yeasty.*

After weeks of reporting my increasing sadness, agitation, sleeplessness, and frustration, we decided to d/c Trikafta, waiting until my next clinic visit to check FEV1 and sweat chloride. There is good news: my FEV1 was up by 15 points, the highest since my diagnosis, and my sweat chloride dropped to 49/53 from my baseline of 120. My liver function was fine. Still, I wanted to leap off the Trikafta bandwagon. The psychologist at our CF Center gave me the quality-of-life questionnaire, which confirmed depression. I wonder if others are also struggling.

☐ 10th week: stopped Trikafta

☐ Two days later: *GOOD SLEEP!*

The patient support specialist made a routine check-in call. He sounded surprised that I'd decided to stop. Surprised? I'd told him about my side effects. A reminder call from the pharmacy led to a talk with the pharmacist. I shared why I had stopped taking

Trikafta. He'd heard similar experiences from others and would report the adverse effects. I felt heard.

Our COVID times began. Are the experiences of depression and anxiety from Trikafta or from the fear, uncertainty, and isolation of the pandemic?

2021 was a hard year. I was in the hospital in January, February, and August-September for a total of eight weeks. I almost missed my daughter's wedding. My lung function declined and new cardiac issues arose. I cultured MAC. In September I left the hospital with a pacemaker and a high-risk cardiac med. As we prepared for discharge, my doctor and I decided to try Trikafta again. He suggested I start with half doses. Another one of our CF doctors gave me information about Trikafta "slow start" with titrated dosing. My next first box of Trikafta was delivered to me in the hospital. Everyone was eager to put it in my hands. Feeling apprehensive, I waited and waited to open it as it had been so hard before.

Trikafta, Take Two: October 2021 Through April 2022

I started half doses on October 20th. I experienced new symptoms: urgent and voluminous bowel movements (a/k/a "the purge"), dizziness, and night sweats. I was feeling "revved up." My gut was not happy. Sadness crept in along with fatigue. I became weary. We tried the lower doses with the "slow start," then just the orange "not so happy for me" pills. Everything exacerbated no matter the dose. I continued with a very low dose while experiencing a mix of new and old symptoms: heartburn, exhaustion, bloating, headaches, early a.m. bowel movements (who poops in the middle of the night?), a "buzzy" feeling in my chest then my lips, abdominal pain. I felt a cloud of sadness on my shoulders. My legs are weak, my massage therapist tells me I'm losing muscle tone.

I dream that I am locked in a room that has an elevator, holding a key that doesn't work.

There were so many voices coming my way, encouraging me to persevere. The CF Team: "We want you to get to full strength." And some CF friends: "Keep going, it will get better." It seemed that everyone was thriving on Trikafta.

Clinic visits in February and April once again show benefits from Trikafta.

> "Are the experiences of depression and anxiety from Trikafta or from the fear, uncertainty, and isolation of the pandemic?"

The psychologist gives me the questionnaire and the results show that I am severely depressed. I believe it. My doctor wants to try an SSRI yet most are contraindicated with my high-risk cardiac med. Wellbutrin is okay so I start taking it. Alas, insomnia sets in on top of sleeplessness, increased sadness, and buzziness. One night I feel like I am a rocket ship taking off. After a weekend call with one of our CF doctors I stop Trikafta again. I also stop Wellbutrin.

The CF team wants to help me stay on Trikafta and suggests I work with a therapist to help me cope with side effects and a psychiatrist to find mental health meds that I can take and tolerate. I'm happy for a break. Two weeks later I pass through clinic on my way to the hospital with a CF exacerbation and a significant blockage. My FEV1 has dropped and doesn't improve; it's hard to get a good blow with spirometry. I culture MAC again. In June, we talk about treating the NTM and my doctor tells me the nurse coordinator is in the conference room searching for a modulator I can take. I'm not interested. By July, my exhaustion has lifted and my FEV1 is headed back to baseline. We stopped talking about NTM treatment and CFTR modulators and I start asking about clinical research studies. Ah, ha! There is a silver lining. We who are not eligible for or cannot tolerate CFTR modulators are needed for exciting studies in which those taking modulators are excluded.

I still have questions:

☐ How many others eligible for modulator therapy are experiencing side effects like mine?

☐ Are my gut issues related to Trikafta?

☐ What complementary therapies can help people experiencing mental health side effects and who wish to continue taking Trikafta?

We now appreciate that Trikafta is not a miracle drug for everyone who is eligible to take it. To learn more about our experience, the CFF is interviewing patients who are not benefiting from modulator therapy. An important article has just been published in Current Opinion in Pulmonary Medicine: *Neuropsychiatric adverse effects from CFTR modulators deserve a serious research effort*. THANK YOU to authors VanElzakker, Tillman, Yonker, Ratai, and Georgiopoulos for this valuable discussion and call for research. A link to the article was shared online with this comment: *It's not you, you aren't alone, your experience is valid.* ▲

Laura Mentch lives in Bozeman, Montana, and is the USACFA historian. She is grateful for the partnership with her CF team and for all the researchers who continue to search for new ways to help us.

A Collective Of Outliers

By Emily Kramer-Golinkoff

The following piece is adapted from a speech Emily Kramer-Golinkoff gave at the CFRI national conference in 2022.

I'll never forget joining the online CF community in 2010—long before the time of TikTok, when Facebook, Instagram, and Twitter were still in their infancy. It was a time when the CF community congregated on old-school forums built specifically for the CF community, composed of rich, intimate message threads, avatars instead of names. We're talking prehistoric internet days here!

It was a place where you could bring your deepest, darkest, most undignified truths and fears, and lay it all out on the table with nothing to hide—a safe haven, and also, sometimes, a terrifying glimpse into the future of what lay ahead. There was love, loss, shame, revelations, validation, and profound, soulful connections. It was a place where many of us "elders with CF" came of age and came to terms with this beast of a disease we were all trying to figure out how in the world to live with—and, in some cases, die from.

This was 2010, two years before the first CFTR modulator would be approved for 4% of the CF population. It was a time when we were all contending with the same killer disease that CF had always been, before we could ever imagine the ways our CF world was about to change—a lifetime ago.

Since then, almost everything has changed. First it was 4%, then it was 50%, and now 90% of the CF community is eligible for a CFTR modulator that radically changes the quality and trajectory of so many people's lives.

We've experienced a pregnancy boom! People have gone to graduate school and launched new businesses! They've run marathons, traveled the world, and started dreaming of a future that previously felt too dangerous to even entertain. For once, people have gained the freedom and space to think about things other than CF! If all of that weren't enough, what's maybe most wild to me is that they've started to see their lung function go up, defying the inevitable descent of CF, for the first time in decades—or maybe even ever.

It's been mind boggling to witness. I feel overcome with pride for our community with each new accomplishment people achieve. My heart swells as I see people's true spirits, character, and talents start to emerge now that the heavy fog of CF has lifted, even just a bit. It's crazy what you can do when your days —and your brainspace—are not entirely consumed by CF.

EMILY KRAMER-GLOINKOFF

For me, that concept is still a thought experiment though, still just a pipedream. With 30% lung function and two copies of a nonsense mutation, I am part of the "final 10%" that doesn't benefit from any of the currently available CFTR modulators. I am still swimming against the current, paddling my hardest, fighting the natural progression of this disease with all of my might, and still coming to terms with the gut wrenching realization that no amount of discipline or hard work, will or love can effectively halt the inevitable progression of this beast of a disease.

> "The hopeful advances that were coming down the pike weren't going to benefit people with nonsense mutations like me."

That is our dear old CF—rabid, vile, and unthinkably unfair.

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So back in 2011, I experienced the collision of two powerful but opposing forces. The first: For the first time in CF history, there was real **hope**. CFTR modulators were in development that were poised to revolutionize the face and the trajectory of this disease. And the second: **desperation**. Despite my best efforts, my disease was rapidly progressing. My lung function hit the 30s, I was diagnosed with CF-related diabe-

Continued on page 28

PHOTO BY STEVEN HOLLOWAY

Life-Mast

Alveoli:
Tiny sacs, tied together
Knotted in place with muscles
Wrapped by bones
Mummified in skin
Penetrated by cystic fibrosis
Lashed fore and aft
in anger

What was I thinking, by commanding this lashing
with angry ropes to the mast of my life-ship
that I might hear death more keenly?

Why, in a rage, did I stop up the ears of my comrades
that I might hear death more keenly?
Now they can't hear me, nor I them. For they are dead.

In proud anger, I demanded the cords be drawn tighter
that I might hear death more keenly.
Now the Sirens of anger sing louder than the Sirens of
death.

Where can I flee their sinewy notes
And strive again to hear more keenly?
Oh, that's right, I can't flee. I'm bound. To the life-mast.

Here:
penetrated by cystic fibrosis,
mummified in skin,
wrapped up by bones,
knotted in place with muscles,
tightly tied,
all the alveoli

They're crowded within, fast becoming a crowd of dead,
my own Hades.
If only I had known earlier how to listen to their
melodious inspirations
and expirations.

-C. Dunafon, 2008

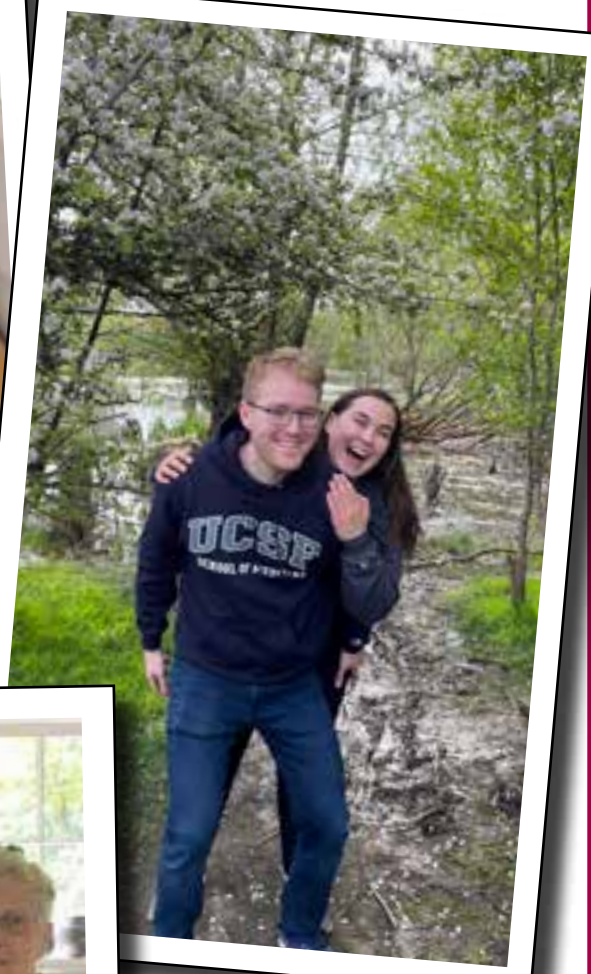
FROM OUR FAMILY PHOTO ALBUM...

MELISSA AND BRIAN TEEMAN WITH THEIR THREE PUPS (LEFT TO RIGHT): CAMMIE, DENNIS, AND BO, CELEBRATING MELISSA'S 19TH LUNG TRANSPLANT ANNIVERSARY IN LAKE TAHOE.

ADAM KEYS AND SYDNA MARSHALL CELEBRATING HER 43RD BIRTHDAY THIS SEPTEMBER AT THE MARINA IN BARCELONA, SPAIN.

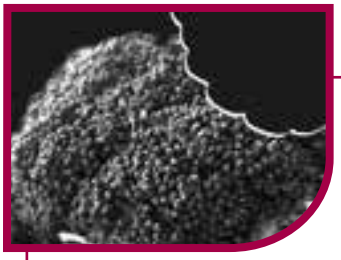
COLLEEN AND SCOTT ADAMSON ON THE RIVER WITH PENNY.

CAMILLE RICHARDS WITH HER NIECE AND NEPHEW.



JACOB GREENE AND JACLYN HODGSON.

THE FAMILY PHOTO IS WITH MY SIBLINGS TAKEN THIS SUMMER IN ASHEVILLE NC. LEFT TO RIGHT (YOUNGEST TO OLDEST): LAURA, CHRIS, JULIE, MARTHA, SUE.



CULINARY CORNER
Vegan Sweet Potato And Black Bean Chili

By Maggie Williamson

As I sit here writing this, I am looking out my apartment window and I can definitely say fall has arrived. The wind is gusting and the sky is like a sheet of grey with no discerning features. All I can think about is warming comfort food and my vegan chili came to mind. I love beef chili, don't get me wrong, but I came up with this recipe a few years ago when I was trying to add more meat-free meals into my rotation. It is a complete meal on its own, but pairing it with corn bread or rice is also delicious! Bon appétit!

MAGGIE WILLIAMSON

Vegan Sweet Potato And Black Bean Chili

Yield: 4 servings

Ingredients:

2 tbsp olive oil
1 medium-sized white/yellow onion, finely chopped
3 cloves of garlic, minced
2 tsp ground cumin
2 tsp ground coriander
2 tsp oregano
1-2 tsp chili powder (depending on your preferred spice level)
1-2 chipotle chilies, chopped finely (depending on your preferred spice level)
2-3 medium peeled and chopped bite-size sweet potatoes
1 15oz can black beans
1 15oz can diced tomatoes
1 ½ cups vegetable stock
2 tbsp tomato paste
Salt and pepper to taste

Optional Garnishes:

Chopped cilantro
Green onions
Sour cream
Feta cheese

Preparation:

Step 1:

Add olive oil to a deep-sided pan/pot on medium heat. Add chopped onion and sauté until translucent, 2-3 minutes. Add chopped garlic, spices, and chipotle peppers. Stir and let cook for another 2 minutes.

Step 2:

Add in sweet potatoes and give it a stir. Add the whole can of black beans, including the liquid. Add the canned tomatoes, vegetable stock, and tomato paste.

Step 3:

Stir it all together. Let it come up to a boil and then turn the heat to low and let simmer for around 20 minutes. Check that the sweet potatoes are fork tender. If you want a thicker chili, you can continue to let it simmer until more liquid is evaporated. Serve with any and all toppings. Enjoy!

*Suggestion:** If you don't have all the spices, you can buy a packet of chili seasoning and use that instead.

Maggie Williamson is 35 years old and has cystic fibrosis. She received a double lung transplant in 2014. She now lives in the U.K. with her British husband, Tom, and their Bengal cat, Charlie. You can find her and all of her cooking delights on Instagram @justasprig

Scholarships Offered By USACFA

USACFA proudly offers three different scholarships! Both the Scholarship for the Arts and the Higher Education Scholarship were set up in memory of a loved one. Our newest scholarship kicks off this fall with a one-time deadline of 4/30/2024. You may apply for more than one scholarship each year, but you may only be awarded one per academic year. If you do not win, your application can be moved to the pool of applicants for another relevant scholarship in the same cycle. For questions about future scholarships or anything related to the application process, please contact us at scholarships@usacfa.org.

Scholarship for the Arts (05/30/24):

This scholarship will award two deserving students $5,000 each toward their tuition in their respective field of the arts: fine arts, computer graphics, design, music, choral, photography, filmmaking, creative writing, poetry, dance, and theater arts, to name a few. It is open to anyone seeking a creative arts degree, whether it be an associate's or a doctorate.

The Scholarship for the Arts was established by Andrea Eisenman to honor her mother, Helen Eisenman. Helen was a single mother devoted to her daughter, Andrea, who has cystic fibrosis.

https://www.cfroundtable.com/arts-scholarship

IN MEMORY OF
LAUREN
MELISSA KELLY

Higher Education Scholarship (06/30/24):

The Higher Education Scholarship was set up by Nancy Wech, in memory of her daughter, Lauren Melissa Kelly. The academic scholarships of up to $2,500 are awarded to two adults with cystic fibrosis who are pursuing career certifications, associate's, bachelor's, and graduate degrees.

Any student seeking a degree in higher education, from associate's to doctorate, is welcome to apply. We look for students who demonstrate tremendous academic achievement, community involvement, and a powerful understanding of how their CF—matched with these achievements—places them in a unique situation to gain leadership roles within the community.

https://www.cfroundtable.com/highereducationscholarship

The William Coon, Jr.
SCHOLARSHIP

William Coon, Jr. Scholarship (4/30/24):

Any student seeking a degree in any of the following is welcome to apply: business, economics, communications, political science, information, project management, finance, accounting, public administration, or marketing. We believe that any higher education is a strong foundation for advocacy and involvement in the CF community.

William J. Coon, Jr. established $20,000.00 in scholarship funds to be awarded in $2,500.00 scholarships for four students each year over a period of five years, totaling 20 scholarships. Mr. Coon was both a cystic fibrosis patient and a businessman who valued the importance of education and "paying it forward."

https://www.cfroundtable.com/williamcoonjrscholarship ▲

Are you interested in establishing a memorial scholarship honoring a loved one from the CF community who has passed away? Please reach out to us at scholarships@usacfa.org to learn more. A member of our Scholarships Committee will follow up with you promptly!

Encourage Family and Friends to Sign Donor Cards

Give the gift of life that lives after you.

To receive donor cards, call:

United Network For Organ Sharing 888-844-6361

Announcing The Recipients Of The Arts Scholarship

The U.S. Adult CF Association (USACFA) is pleased to announce the recipients of the Scholarship for the Arts, offered in memory of Helen M. Eisenman. We offer this arts scholarship in Helen's memory—she was a Holocaust survivor with a passion for the arts. A talented photographer, she eventually earned a reputation as the "Doyenne of Subtitles" within the film industry for her skills in subtitling films in multiple languages. She made many sacrifices over the years so that her daughter, Andrea, who has cystic fibrosis, could live as long as possible. Helen always encouraged Andrea to be creative, read books, appreciate museums, and listen to music.

In our evaluation, we look for students who demonstrate tremendous artistic creativity, originality and achievement, community involvement, and a powerful understanding of how their CF—matched with their creative endeavors—places them in a unique situation to impact the world through their art. The scholarship is open to anyone seeking a degree, from an associate to a doctoral degree, in the creative arts: fine arts, computer graphics, design, music, choral, photography, filmmaking, creative writing, and poetry, to name a few.

We are pleased to announce **Grace Lidgett** and **Stella Guthman** as the recipients of this year's arts scholarships. They were each awarded $5,000. Congratulations to both!

GRACE LIDGETT

STELLA GUTHMAN

Grace Lidgett is currently attending Grace College in Winona Lake, Indiana. Grace plans to become a graphic designer upon graduation. In her own words: "Through art, we can invite one another into a small section of our lives with every creation, and, more specifically, I can do that through the portrayal of hardships and hopefulness." While she enjoys using many different mediums in creating her art, Grace prefers to use charcoal: "The charcoal at first is full of chaos, but after I am able to take a step back and realize the artistry within the insanity, something striking comes from it. I can discover an abstract image within the shapes that I enhance and finalize. I use this process to depict themes of beauty and suffering, and I portray through the artwork the feeling of hopefulness in a tough situation. Just like when I step back to look at the canvas, I do the same with my own life. When I view my life from a new perspective, I see sprouts of hope and love around every corner, and those are the things that I love to physically recreate on canvas and paper."

Stella Guthman is currently enrolled at the University of Nebraska at Omaha. She aspires to be a graphic designer and magazine layout editor for NASA upon graduation from college. She has always wanted to be an astronaut for NASA but realized it would be too much of a burden with cystic fibrosis. Stella later learned that, as a graphic designer, she can take the images from NASA and manipulate them so anyone can feel like they are right there with the astronauts in space. A new dream was born—she can use her artistic skills and apply them to science!

Scholarships are offered in the spring semester each year. More information, including the application and relevant deadlines, can be found on our website. For questions about future scholarships or anything related to the application process, please contact us at scholarships@usacfa.org. ▲

BE SURE TO CHECK US OUT ON SOCIAL MEDIA:

FB CF Roundtable: www.facebook.com/CFRoundtable and
FB CF Connect: www.facebook.com/groups/cfconnect
Twitter: https://twitter.com/CFRoundtable
Linked In: https://www.linkedin.com/company/us-adult-cf-association-usacfa-

Recipients Of The Higher Education (Formerly The Lauren Melissa Kelly) Scholarship Announced

The U.S. Adult CF Association (USACFA) is pleased to announce the recipients of the Higher Education (formerly the Lauren Melissa Kelly Scholarship).

In our evaluation, we look for students who demonstrate tremendous academic achievement, community involvement, and a powerful understanding of how their CF—matched with these achievements—places them in a unique situation to gain leadership roles within the community. Our scholarship is open to all pursuing any degree, from associates to Ph.Ds. We believe that any higher education is a strong foundation for advocacy and involvement in the CF community.

Nancy Wech established this scholarship in honor of her daughter, Lauren Melissa Kelly. This semester's winners demonstrated outstanding potential, just like Lauren years ago. Lauren was an inspiration to all who knew her. An incredible leader and scholar, her drive and success are the foundation of her memory. She was transformative in every aspect of her life. She had distinguished herself as a member of the Golden Key Honor Society, Mortar Board, Phi Upsilon Omicron, Gamma Beta Phi, Delta Gamma sorority, and was chosen as one of ten Senior Leads at the University of Georgia. She acted as one of the re-founding members of the Phi Kappa Literary Society and was significant in the metamorphosis of the Z Club into the William Tate Society. Although Lauren lost her battle with cystic fibrosis late in her senior year, her hard work and memory continue to live on through her inspiring involvement.

We are pleased to announce our winners of the scholarships for this calendar year. They will each be awarded $2,500. Congratulations to both!

At the age of 27, after getting divorced, Jenny decided to go to college. This older student mindset has helped her tremendously in pursuing her academic goals. She is currently enrolled at the Utah State University School of Graduate Studies where she is working on completing her masters in social work. Additionally, Jenny holds an undergraduate degree in psychology from Utah State University. Jenny previously worked as a freelance writer and online community manager for Bionews Services, Inc. Jenny has been active with her local CF center and helped establish a patient advisory board for adult cystic fibrosis patients in Utah and welcomes the challenge and joy of thriving in leadership roles.

Our second recipient is working on a master's degree in philosophy and anthropology with a focus on decolonial and anticapitalist theory and practice. They're an herbalist and nutritionist by trade, specializing in CF. They recently received a double lung transplant.

Both scholarship winners demonstrated the leadership, intelligence, and drive of Lauren Melissa Kelly. All of us at USACFA look forward to seeing them further develop their leadership and advocacy in the cystic fibrosis community.

Scholarships are awarded each year. More information, including the application and relevant deadlines, can be found on our website. For questions about future scholarships, or anything related to the application process, please contact us at scholarships@usacfa.org. ▲

LECOINTRE continued from page 7

classification, of which 7,591 children had a clinical diagnosis of CF and 1,174 had a clinical diagnosis of CRMS. CRMS patients exhibited normal nutritional indices and pulmonary function up to age 9-10 years. The presence of respiratory bacteria associated with CF, such as *Pseudomonas aeruginosa* from CRMS patients ranged from 2.1-9.1% after the first year of life. In conclusion children with CRMS demonstrate normal pulmonary and nutritional outcomes into school age. However, a small percentage of children continue to culture CF-associated respiratory pathogens after infancy.

https://tinyurl.com/25ynztfk

Dietary Intake, Weight Status, Pulmonary Function, And Metabolic Profile In Children With Cystic Fibrosis With Or Without Pancreatic Sufficiency

Nutritional status and growth is well associated with disease outcomes and lung function in patients with cystic fibrosis. Current dietary guidelines for the management of CF recommend a high-calorie, high-fat diet. Pancreatic insufficiency (PI) is present in most patients and contributes to malabsorption and malnutrition, but a number of patients have pancreatic sufficiency (PS). The aim of this study was to compare weight status, clinical characteristics, and dietary intake of children with CF, with PS or PI. Patients with a diagnosis of CF and/or two known muta-

Continued on page 30

tes. I was becoming resistant to available antibiotics, running out of conventional treatment options. The hopeful advances that were coming down the pike weren't going to benefit people with nonsense mutations like me.

It was that collision of hope and desperation that spurred the formation of Emily's Entourage. My family, friends and I decided we couldn't just sit back and let nature take its course. Instead, we dove in, head first, launching Emily's Entourage to speed lifesaving breakthroughs and a cure for the final 10% of the CF community.

At the time, game-changing advances were in development for G551D and the groundswell of focus was shifting to Fdel508 given its prevalence in the CF community. For those of us with nonsense and other rare mutations, there was virtually nothing being done. The science was harder to crack and researchers were singularly focused on much more common mutations. We were not on the research radar; nobody was paying attention.

The worst part wasn't just the lack of treatments in the pipeline, but rather, the lack of hope for future ones, as we quickly realized that there wasn't even an infrastructure in place to get new treatments targeting rare and nonsense mutations into the pipeline. And so, we set out to build a "foundation of the future" that changes the way—and the pace with which—drug development is done.

From the start, our mission at Emily's Entourage was singular: to bring lifesaving treatments to the final 10% of the CF community—and to do it fast. We were not interested in recreating the wheel. Our goal was to fill critical areas of unmet need and to operate quickly. We sought to support work that otherwise wouldn't happen or to make work happen faster than it otherwise would.

This was all new to us. We had no idea what we were doing, but what we did have is a crystal clear vision of where we wanted to go. What we lacked in knowledge, we more than made up for with passion, laser focus, and tireless hard work and it turns out, having an "outsider" status can actually be an asset to innovation. We leveraged our outsider status to see things in new, fresh ways and reimagine the way research and drug development could be done.

Ten years later and we have come a long way!! Among our highlights, we've:

- Raised over $11M
- Awarded 32 research grants to top academic institutions around the world and secured $42.8 M in follow on funding
- Played a pivotal role in advancing multiple research projects to preclinical and clinical stage, including Spirovant, the now-acquired CF gene therapy company that EE launched through venture philanthropy, and cystetic medicines.
- Developed 14+ phage therapies to potentially treat Methicillin-resistant Staphylococcus aureus (MRSA) and nontuberculosis mycobacteria (NTM)
- Launched a patient registry and clinical trial match-making program to expedite clinical trial recruitment
- Hosted a patient listening session with the FDA to advocate for the urgent unmet needs of the final 10%
- Built a strong, tight-knit scientific community and hosted multiple scientific meetings, including a scientific symposium on gene-based therapy in December 2023.

> "The worst part wasn't just the lack of treatments in the pipeline, but rather, the lack of hope for future ones."

These last few years, we've been racing—leading the charge to close the gap for the final 10%, pushing the boundaries of what's possible. The progress is undeniable and hope is wonderful, but hope alone is insufficient. We need treatments—treatments that reach people with CF and treatments that come fast.

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Back in 2011, we saw the advances coming for G551D and then the scientific community's full attention and resources turned to one thing and one thing only: extending the breathtaking progress to those with Fdel508. Who could blame them given its vast prevalence with 90% of the CF community having at least one mutation?

Science is amazing and brilliant scientists are even more amazing!! I wholeheartedly believe there is nothing they can't do. With the undivided focus of the most talented CF scientific minds and significant financial investment, that hope transformed into reality for roughly 90% of our CF community with monumental approval of Trikafta in 2019—dramatically changing the CF landscape in ways we could have never imagined.

I'll never forget reading the accounts of people taking their first doses of Trikafta, documenting daily updates on Facebook. These updates captured what the data never could:

- The coughing that subsided
- The breathing that deepened
- The hours each day freed from tethering treatments
- The dreams and future, reclaimed
- The "sounds of silence" as one dad poetically wrote

I've never been a cynic. I've always believed in science, but reading those updates, I remember being incredulous at the marvels of medicine—that a little pill could produce these transformations, and sometimes in a matter of minutes! Three pills a day were radically changing the trajectory of this deadly disease right before our eyes.

And just like that 90% of the CF community set off on their historic ascent to better health. For the first time in their lives, they were defying the inevitable descent of CF. Their symptoms were abating, their medical treatments were reducing. They could stand taller. They could run further. They could dream bigger. They. could. breathe.

And here I was on the opposite end of the spectrum, battling resistant lung infections and dwindling lung function, plunging deeper into the cavernous decay of end-stage disease. The feeling of perishing only magnified by the radical transformations so many in the CF community were experiencing around me.

Up vs. down.

Hope vs. no hope.

Life vs. death.

It was like our CF community had been divided into two: the haves and the have nots—90% with unprecedented, unbridled hope, and 10% with nothing. Helpless, hopeless, and running out of time, this was rock bottom and it was an excruciating place to be.

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People often describe the emotion of not benefitting from CFTR modulators as "bittersweet," but for me, that description has never exactly captured it. Even in the midst of my desperation and heart ache, I was so happy for my CF friends who benefited. My happiness was pure and unadulterated. There was nothing bitter about the sweetness. I wouldn't have traded the advances for anything. I just desperately yearned for the rest of us to share in the progress too.

Still, there's no denying that the approval of the modulators fragmented the CF community, creating massive disparities in health status, treatment needs, access, and prognosis. No longer were we one, uniform group contending with the same disease. Suddenly, we were fractured into thousands of CFTR subpopulations, each with a radically different variation of this disease and trajectory.

While we often talk about the "final 10%" that was left behind, the truth is that 10% is actually not accurate. It is a vast underestimate. The reality is 10% only refers to individuals with genetic mutations that are ineligible for CFTR modulators, but there are many other subpopulations that don't benefit either:

- People with comorbidities that preclude them from taking modulators.
- People who develop intolerable side effects and have to stop modulators.
- People who do not respond to modulators.
- People who are post transplant for whom modulators came too late.
- People who live in parts of the world who don't have access to modulators.
- And lest we ever forget, people who died far too early because they never got the chance to try.

In fact, within Asian, Black, Hispanic, and Native American populations, the number is considerably higher than 10% with roughly 17-40% of people not benefitting from modulators, which only further compounds existing systemic issues that lead to health inequality.

Those of us who don't benefit from currently available modulators, we are "the outliers"—a collective of many outliers, left behind by the breathtaking progress, stuck on the outer cusp of science. It's exactly the place you never want to be.

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While being part of the group left behind was undeniably a tough pill to swallow, it didn't dampen my happiness for those who benefitted or my utter awe at the miracles of science. Instead, it fueled it. As if radically transforming the lives of those that benefitted—providing huge improvements in lung function, reductions in frequency and severity of infections, and even cutting the number of people needing lung transplants by two thirds—wasn't enough, more than anything, what the modulators have done is shown us what's possible.

After the big promises made in 1989 that gene therapy would provide "cures within 5 years," our community was jaded, for good reason. The modulators have proven what science can do. They have turned us into believers again, and in doing so, they've whet our appetites even more. And that's a good thing because we've come so far, but we are not there yet, not even close.

- Not for the 10% that is ineligible for modulators due to their mutations, whose lung function and quality of life continue to slip away.
- Not for the people with comorbidities or intolerable side effects, who got tantalizing close to their breakthrough, only to have them snatched away
- Not for the non responders or those that are post transplant, contending with life threatening infections, rejections, and cancer
- Not for those who still don't have access to modulators, who are continuing to lose their lives while a lifesaving medicine exists but are just beyond their reach

The progress is remarkable, but it isn't good enough. That was our motivation for starting Emily's Entourage 10 years ago and that is my motivation for devoting my life—my waning time and breath—to securing a better future for 100% of the CF community, with nobody left behind. That is my life's work.

There are thousands of people just like me, vying to return to careers and start families, to run marathons and explore the world, to plan for a future they actually believe in, to start living a life that isn't entirely dictated by this beast of a disease. They deserve better and it's up to us to get there.

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For so long, my greatest fear was that a modulator would come for the 90%, it would provide long-awaited relief, and then poof, everyone would disengage. Who could blame them? CF

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SALTY PARENTING
Caffeinate Then Advocate

NEW COLUMN

By Katie Lockwood

I walk into the children's hospital and I don't need to ask anyone for directions. I know my way around.

I have my handicap placard and choose valet parking—I'm fancy and there is a discount.

I get to the appointment and check in. Name. Date of Birth. Why am I here?

They pass me paperwork to review and sign with a likely questionably clean pen.

"Take a seat in the waiting room," they say.

I put on my big girl pants. Advocacy mode on! I have cystic fibrosis; I cannot be in the waiting room.

Huh? Everyone looks confused. They look in the portal. Your daughter?

I have CF; my little one does not. **Yes—this is her appointment.** However, I cannot wait in the waiting room. We need to be placed in a private room.

I'm already exhausted from this exchange. This is a frequent occurrence. One of my little ones needed hip surgery this past spring. I was told we would spend the night in a shared room. Umm what?!? We are in a pandemic and sharing a room? I have cystic fibrosis—I cannot stay over in a shared room.

I became robotic with each person in the rotation. I have cystic fibrosis. Hospital protocols; private room required. Will add to the note. Yup, sounds great. During the procedure they try to place us in the group waiting area. We grab a bagel and sit in the fake garden instead, while we wait for the call that the surgery is complete.

I bemoan internally that I need to share my condition with everyone I speak to. I'm so tired. Did I mention my little one had surgery? I'm so, so tired.

KATIE LOCKWOOD

Parenting with CF is a fairly new phenomenon. The little ones that we now have will have appointments and procedures and their own medical problems—hopefully not too many with hip SPICA casting as that's *no fun*!

There will be medical issues, though, and many waiting rooms that need to be navigated. We are in the beginning of a new era for cystic fibrosis and I look forward to the policies that will need updates so that we may not always need to be an open and loud book in order to stay healthy. Until then, I will try my best to have time to get my cappuccino so I can caffeinate, then advocate. ▲

Katherine Lockwood is 35 years old and has CF. She lives on Cape Cod with her husband, Arden, and her girls, Rose and Magnolia. She is a therapist for Verge Therapy *and focuses on supporting individuals and couples experiencing disability. She is the author of* Why Me, Mama? *an award-winning children's book about the disability experience. You can follow Katie's picture book projects at Acorncottagepress.com and on Instagram @acorn_cottage_press.*

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tions for CF, ages 1 to 19 years were included in the study. Weight status, pulmonary characteristics, and blood lipid concentrations were evaluated. Dietary intake was evaluated through four 24-hour recalls and energy, macronutrient intake, and intake in terms of food groups were assessed. 134 patients with CF were included in the present analysis. The percentage of overweight/obesity was higher in PI than in those with PIm 47% vs 22%. Overall children with PS had a higher body mass index, blood lipid levels and pulmonary function tests. Total energy intake was lower in children with PS vs PI. Weight status, dietary intake, pulmonary function, and lipid profile differed significantly in children with CF by pancreatic status. To avoid obesity, dietary recommendations for a high-calorie, high-fat diet should be reconsidered in patients with CF regarding their pancreatic status.
https://tinyurl.com/4us8bdzk

Novel CFTR NBD1 Stabilizers Correct ΔF508-CFTR Domain-Domain

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is traumatic and brutal. After being so tightly tethered to CF for so long, who wouldn't jump at the chance for some distance, to take a little breather, to *finally* live a life that's not entirely dictated by CF?

But what I can tell you is these past few years, I've been buoyed in ways I can't describe in words by people with CF and their families who have stepped up, raised their hands, and taken on this final chapter of the quest as their own. They've committed their time and energy, their talent, connections, and financial support to "the final 10%" and our work at EE. They've rallied behind our efforts to raise funds for research, to share our surveys, to join our patient registry and encourage others to do so too.

On a personal level, they've rallied behind me too, uplifting me during some of the toughest times and reminding me that I'm not alone or forgotten. Upon marveling at the ways Trikafta has changed my friend's life, she responded to me, "It won't ever feel right until the 10% get to do the same." Fragmented by mutations; united by heart and soul—that is the spirit of the CF community. That's the force that pulled me in when I first joined the online CF community in 2010. It's the power of a community. While a lot has changed in the past 13 years, one critical thing has not—the ride or die, rock solid, bond and solidarity of the CF community remains as intact as ever.

I've learned that what binds us isn't symptoms or medication. It isn't genetic mutations or even shared health traumas and shortened life spans. What binds us is so much bigger, more transcendent than any that. It is a shared history of overcoming the seemingly insurmountable, of persisting against all the odds and carving out lives and dreams despite unthinkable adversity. It's the shared history of finding our voices and creating change. of proving what is possible, and of sticking together through thick and thin.

We have come so far. But we are not there yet, not even close. Now is not the time to let out a sigh of relief. Now is the time to double down, to shift it into high gear, to pool our collective talents and tenacity, to direct our undivided attention and funds to finish the job for the collective of outliers—very single last one and to do it fast. After all, while we may be composed of thousands of different CFTR subpopulations, when it comes down to it, we are one, and we are only as strong as our weakest link.

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This story, our story, is quite the tale. It's a story of breathtaking progress, of heartbreaking pain, of incomprehensible inequality, of transformation and innovation, of heart and soul, and it is a story that's as of yet unfinished. It won't be done until we have lifesaving breakthroughs for every single person with CF —every single mutation, every single person around the world, every single outlier, with nobody left behind. So, my friends, let's get going. Because we have one heck of an ending to write... and my goodness, it is time for this CF story to finally be complete!

To learn more, visit emilysentourage.org and @emilysentourage on social media.

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Emily Kramer-Golinkoff is Co-Founder of Emily's Entourage, an innovative 501(c)3 that accelerates research for individuals in the final 10% of the cystic fibrosis (CF) population who do not benefit from existing mutation-targeted therapies. She is also an internationally recognized patient advocate and speaker.

Since 2011, Emily's Entourage has awarded millions of dollars in research grants, launched a now-acquired CF gene therapy company, developed a patient registry and clinical trial matchmaking program to accelerate clinical trial recruitment, and led worldwide efforts to drive high-impact research and drug development. The organization has been featured in media outlets, including New York Times, STAT, CNN, Yahoo, AOL, People, The Philadelphia Inquirer *and more.*

Emily has a master's degree in bioethics and certification in clinical ethics mediation from the University of Pennsylvania, where she also completed her undergraduate degree. She has given talks at The White House, TEDx, University of Pennsylvania's Annenberg School for Communication Commencement, Stanford University's Medicine X Conference, and more. Emily was named a "Champion of Change" for President Obama's Precision Medicine Initiative and is the recipient of the 2020 Philadelphia Magazine Luminary Award and the 2016 Global Genes Rare Champion of Hope for Advocacy Award. Learn more at emilysentourage.org. ▲

Assembly Defects

ΔF508 is the most prevalent mutation detected in patients with cystic fibrosis (CF), and it causes a loss of F508 within CFTR's first nucleotide binding domain (NBD1). Researchers from Sionna Therapeutics Inc. recently reported the discovery and preclinical evaluation of novel small-molecule CFTR NBD1 stabilizers and CFTR assembly correctors as potential new agents for the treatment of CF.
https://tinyurl.com/48xe99dn

A Glycosylated Hemoglobin A1c Above 6% Is Associated With A High Risk Of Developing Cystic Fibrosis-Related Diabetes And A Lower Probability Of Weight Gain In Both Adults And Children With Cystic Fibrosis

The classical glycosylated hemoglobin A1c threshold of 6.5% is an insensi-

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IN THE STOPLIGHT
With Jacob Greene

By Andrea Eisenman

Age: 24
Home: Edmonds, WA
Current Location: San Francisco, CA

Readers, it is my pleasure to introduce Jacob Greene, who recently joined our board as a director. You may have seen his photo in recent issues because he has received the Higher Education scholarship, not once but twice. He won initially as an undergraduate and the second time while pursuing a medical degree at the University of California San Francisco (UCSF). Jacob became a director in spring 2023 and is active on several other CF boards. As you will see when you read his interview, he is highly motivated to keep himself healthy and become a caring physician due to his personal experience as someone with a lifelong illness. Since starting Trikafta in 2019, his health has improved immeasurably and he is able to tackle more effortlessly all that lies ahead for a bright future. This propels him to make things better for others once he is an anesthesiologist. He hopes to ease his patients' fears and anxieties and possibly contribute to making lives better for those in need of medical assistance, as recent medical innovations, such as modulators, have done for him. Please welcome our latest star. Spotlight, please!

Where did you attend school for your undergraduate degree?

I attended Stanford University from 2017-2021 and I hold a Bachelor of Science with honors in Biology.

When were you diagnosed?

I was diagnosed at birth after an ultrasound revealed that I had meconium ileus.

Was your sister also tested at birth due to your diagnosis? Where is she now?

JACOB GREENE ON THE SLOPES.

Since my family had no history of CF, my parents did not know they were carriers until I was born. My sister was diagnosed with CF via amniocentesis before being born. My sister recently graduated from the University of Washington and now works in San Diego, CA, at Bristol Myers Squibb as a research associate.

How was it growing up with a sibling with CF?

Both my sister and I are homozygotes for F508del. Growing up before Trikafta, we were fortunate to be relatively healthy. As a kid, I was chronically colonized with *Pseudomonas*, while my sister frequently cultured *Staphylococcus aureus* and *Aspergillus*. Despite us living together, we seldom cultured the same bacteria. Now, both my sister and I are on Trikafta. Neither of us has needed any IV antibiotics since starting Trikafta in 2019. I have not cultured any bacteria since starting Trikafta.

Where are you studying medicine?

I am a medical student at the University of California San Francisco (UCSF) School of Medicine. UCSF does not have an undergraduate college, so many people outside of the Bay Area have not heard of UCSF. However, UCSF is the major academic medical provider for San Francisco. UCSF also operates the San Francisco Veterans Affairs Hospital and San Francisco General Hospital.

How is medical school so far? What is the most challenging?

I just finished my first year of medical school and am starting my second year. So far, it has been great! I have learned a ton and am excited to care for patients full time starting in January of 2024! I think the most challenging part of medical school is how different it is from high school and college. After the one-and-a-half years of pre-clinical curriculum, we are learning to care for patients in the hospital full time for the remaining two-and-a-half years. Thus, the types of accommodations needed for this clinical curriculum differ from classroom learning. For example, my academic accommodation is that, when I am around patients in the hospital, I wear an N95 mask. Thankfully UCSF, has been incredibly accommodating of this. Additionally, since my CF center is also at UCSF, it makes getting to my doctor appointments very easy.

What made you choose UCSF?

There are several reasons why I

chose UCSF. My girlfriend, who is a genetic counseling student, matched at UCSF for genetic counseling school. When she matched, that largely sealed the deal. But additionally, UCSF was the most financially affordable option for me. I also was a patient at UCSF when I lived in the Bay Area from kindergarten through half of third grade. My first memories as a CF patient were from UCSF. I remember my first inpatient hospitalization, which was at the UCSF Parnassus campus. So UCSF holds some sentimental memories for me, too.

How do you balance your needs versus your work and time for your relationship?

Ultimately, everything is about priorities. I prioritize my relationship and taking care of my health because, at the end of the day, I can learn and, eventually, care for patients only if I am caring for myself. If I am sick, then I can't effectively learn or treat patients.

Do you exercise regularly?

Yup! I run a couple of miles three times per week.

Did you always want to be a doctor?

Initially, my inspiration to be a physician was due to my experience as a CF patient. From birth until the age of 20, I consumed over 250,000 pills, spent 10,000 hours doing medical therapies, received over a dozen rounds of inpatient intravenous (IV) antibiotics, and was operated on more than ten times. Before Trikafta, this was a pretty typical representation of life with CF. These experiences as a patient have inspired me to be a doctor. Since starting Trikafta, my FEV1 has completely stabilized, I have not needed any IV antibiotics, and my treatment regimen has pretty much been eliminated. I want to be a physician to give the gift of medicine to others. Furthermore, I believe my first-hand experiences as a patient will inform my clinical practice. There are not many people with CF who are physicians, so I hope combining my experiences as a patient with my ongoing medical training will help me be the best physician I can be.

How were your undergraduate studies? How was your health through those four years?

I was hospitalized every 12 to 18 months for CF infections during college and, despite my FEV1 being in the 80s, my health was getting worse. Then, in December 2019, I started modulators. Amazingly, there was no longer a need for IV antibiotics. I still occasionally use them if I have a cold, but my daily treatment regimen is very minimal. I have not cultured *Pseudomonas* since starting modulators.

Do you know the area of concentration you will go into after medical school? If so, why?

I am pursuing a residency in anesthesiology, although one does not apply for residency until the fourth year of med school. I like the physiology, pharmacology, and hands-on nature of the specialty. Patients are often nervous going into surgery, as I have been when I was a patient. Anesthesiologists can help alleviate some of that anxiety. I vividly remember my anesthesiologists and wanting to provide the excellent care I have received motivates me to pursue this specialty. I also am very interested in the sub-specialties that branch off anesthesiology—critical care medicine, pain medicine, neuroanesthesia, or pediatric anesthesia.

You received the Lauren Melissa Kelly Higher Education Scholarship from USACFA. Tell us about that.

Yes, twice. Once as an undergrad and once in med school. Medical students graduate with an average of $200,000 in student loans, so the scholarships I have received go to offset my student loan burden.

Do your friends and classmates know you have CF? Do your professors know?

I am very open about my CF. I take enzymes publicly. My professors know about my CF because the disability office shares that information with them. One unexpected benefit of being so open about my disease is I have been invited to give talks at school about CF. Every year, I give a lecture about life with CF at a class taught at Stanford University (my alma mater). Now, I lead a Q&A session with the UCSF Genetic Counseling students about CF.

Who or what are sources of support for you in times you need it?

My family and friends are my main source of support.

Now that you are on Trikafta, what is most important for you to keep doing treatment-wise to stay healthy?

For me, besides taking Trikafta and pancreatic enzymes, regular exercise is most important. I am not particularly nervous about starting my clinical years of med school.

Do you have advice for others who have CF who may consider medical school?

The first thing I would say is that representation is very important! There are not many physicians who have cystic fibrosis. We (CF patients) have valuable insight into what it is like to be a patient. And with medications like Trikafta, now is as good a time as ever to pursue a career as a physician. I would also advise those with CF (or any other chronic illness) starting medical school to connect with more senior medical students at their school to get their advice on how to do well academically. I also would recommend plugging in with the school's disability office if any accommodations are needed. Medical education is very different from primary school/high school/college.

Did you have a mentor or someone

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Remembering Isabel Stenzel Byrnes

(1972 – 2023)

Isabel Stenzel Byrnes, L.C.S.W., M.P.H., a remarkable woman of grace, wisdom, strength, and compassion, passed away on July 12, 2023. She was 51 years old, 19 years post-lung transplant, and had recently celebrated her 25th wedding anniversary with the love of her life, her husband Andrew.

Isa wrote the *CF Roundtable* column, Spirit Medicine, for the past 17 years. Readers often mentioned that upon receiving their issue of *CF Roundtable*, they turned to Isa's column first. Isa imparted her knowledge and strategies for living a full life with CF, as well as ways to deal with the challenges life with CF can pose.

Isa was also a member of the *CF Roundtable* Speakers Bureau, where she informed audiences of the lessons they could learn from her life experiences. Isa was an accomplished speaker presenting at national conferences around the country and abroad. Her talk at the CFF Volunteer Leadership Conference in 2023 led to rave reviews from those in attendance.

ISA STENZEL BYRNES

Isa was the keynote speaker at the *CF Roundtable* event held at the CF Foundation North American Conference in November of 2019. She spoke about resilience as over one hundred attendees sat mesmerized by her insight and sage advice. There was not a sound in the ballroom as Isa spoke about finding ways to be resilient even when experiencing adversity. Her 2022 *CF Roundtable* webcast, Writing for Healing, continues to be an invaluable resource for people with CF and their families.

Isa's 2014 TED talk, "The Art of Saying Goodbye," has been viewed over 135,000 times. Many people in the CF community and beyond have watched the talk in the past month to listen to Isabel's guidance on dealing with the loss of a loved one.

Isa received a double lung transplant in 2004. Isa and her twin sister Ana, who also had CF, wrote a book, *The Power of Two–A Twin Triumph Over Cystic Fibrosis* (Univ. of Missouri Press 2007), and traveled around the country and abroad speaking at book events filled with their fans. *The Power of Two* inspired a documentary about their

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tive screening test for cystic fibrosis-related diabetes (CFRD). They sought to identify CF-specific A1C thresholds associated with 1) risk of progression to CFRD and 2) changes in body mass index (BMI) and forced expiratory volume (FEV1). For the onset of OGTT-defined CFRD optimal A1c threshold was 5.9% in adults and 5.7% for children. Kaplan-Meier analysis of progression to CFRD according to baseline A1C showed increased the risk of developing CFRD for A1c ≥ 6.0% in adults and ≥ 5.5% in children. Temporal changes in BMI and FEV1 according to baseline A1C in adults were assessed with a linear mixed-effect model, BMI significantly increased over time in subjects with a baseline A1c < 6%, but those with a A1C ≥ 6.0% gained significantly less weight over time. There was no difference in FEV1 according to baseline A1c category.

https://tinyurl.com/3rc9fyda

Real-World Outcomes And Direct Care Cost Before And After EelexacaftorTtezacaftor/Ivacaftor Initiation In Commercially Insured Members With Cystic Fibrosis

Elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) is a triple combination oral drug therapy with an annual cost greater than $300,000 and available to nearly 90% of the CF population based on age and genotype. Limited real-world direct medical cost offset data are available for ELX/TEZ/IVA among commercially insured individuals. Among members with CF newly initiating CFTR modulator with ELX/TEZ/IVA, mean member total cost of care increased 3-fold despite significant and meaningful reductions in pulmonary exacerbation events, HRU, and medical benefit spend. Pharmacy benefit spend outpaced medical benefit spend at a rate of $9.64 to $1 in the 180 days following ELX/TEZ/IVA initiation. Real-world data should be used to objectively measure the clinical and economic benefits of costly medica-

lives which screened at 30 film festivals globally and won ten festival awards. It has been seen and appreciated by thousands of people over the years.

Several people with CF who were living in Japan reached out to both Isa and Ana after reading their book and learning of their half-Japanese/half-German heritage. The letters they received alerted them to the lack of treatments available in their country. Because CF is not common in Japan, the sisters saw the need to increase awareness throughout the medical community about the lack of treatment options. *The Power of Two* movie includes footage of Isa and Ana organizing community members. Their efforts allow CF medications to be provided to Japanese children and adults with CF, ultimately saving lives. Isa's reach went beyond the CF community. She touched the lives of many through her work as a bereavement social worker for Mission Hospice in the Bay Area for the past eight years. She led support groups for family members who had lost a loved one, taught writing courses, and helped families deal with loss.

Isa was also a dedicated volunteer with CFRI. She led support groups, organized conferences, and oversaw the CFRI retreat events for three decades. Isa was an active member of Team CalNev (f/k/a Team NorCal) at the Transplant Games of America and was twice named the Female Athlete of the Year, winning medals in events such as swimming and track and field. It was common to see Isa at the end of the competition day loaded down by so many medals clanking around her neck that you could hear her coming from a block away. She pushed her body to its limits and reaped the benefits of healthy transplanted lungs; so much so that she was also able to become an accomplished bagpiper, which is unheard of for someone with CF. With bagpipe in tow, she participated in many musical events and brought joy to herself and so many others, all while honoring her lung donor Xavier.

Isa was not one to sit idle. When not bagpiping, swimming, biking, or working, she was hiking up mountains or traveling to places on her and Andrew's bucket list. It was her father who loved to hike and took his twin girls camping and hiking on the Pacific Crest Trail at a young age, in the hopes of increasing their lung function. Additionally, these outdoor activities taught her to overcome her fears as she pursued her life's ambitions. She was fearless, which inspired others to try things that may have seemed out of reach for them.

Isa's dedication to helping others with CF had no limit. She was always available to assist a person with CF or their family members. She provided hope and comfort to thousands of people throughout her life. She was a trailblazer who showed people with CF that they could do great things and find meaning in their lives in a variety of ways.

We hold Isabel's beloved husband, Andrew, and her family in our thoughts at their time of grief. Isa's memory is a blessing to the entire CF community. There will never be another person like Isa. She was a brilliant woman with an open and generous heart and an incredible strong spirit. As the *CF Roundtable* community mourns such an immense loss, we commit to continuing her legacy of dedication to helping others and living life, showing kindness and generosity to everyone we meet. ▲

tions, such as CFTR modulators, to align price with value.
https://tinyurl.com/55psp23h

Experience With Elexacaftor/Tezacaftor/Ivacaftor In Patients With Cystic Fibrosis And Advanced Disease

Elexacaftor/tezacaftor/ivacaftor (ETI) was used through the early access program in Spain from December 2019 in cystic fibrosis (CF) patients with homozygous or heterozygous F508del mutation with advanced lung disease. A multicentre, ambispective, observational, study recruited 114 patients in follow-up in 16 national CF units. Clinical data, functional tests, nutritional parameters, quality of life questionnaires, microbiological isolates, number of exacerbations, antibiotic treatments and side effects were collected. The study also compared patients with homozygous and heterozygous F508del mutations. Of the 114 patients, 85 were heterozygous for F508del mutation, and the mean age was 32.2 ± 9.96 years. After 30 months of treatment, lung function measured by FEV1% showed improvement from 37.5 to 48.6, BMI increased from 20.5 to 22.3, and all isolated microorganisms decreased significantly. The total number of exacerbations was also significantly reduced from 3.9 to 0.9. All items in the CFQ-R questionnaire showed improvement, except for the digestive domain. Oxygen therapy use decreased by 40%, and only 20% of patients referred for lung transplantation remained on the active transplant list. ETI was a safe and well tolerated drug, with only 4 patients discontinuing treatment due to hypertransaminasemia.
https://tinyurl.com/ye3wmn9z

Cystetic Medicines Initiates Phase 1 Clinical Trial Of A Molecular Prosthetic For Cystic Fibrosis

Cystetic Medicines, Inc., a clinical-stage biotechnology company developing small molecule channels that serve

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Meet Melissa Teeman

Interviewed by Andrea Eisenman

We are excited to introduce and welcome our newest volunteer, Melissa Teeman. She will assist people with CF who wish to write articles but don't feel they have the tools to do so or perhaps need help crafting their stories. *CF Roundtable* has long been a source of knowledge, community, and support for adults with cystic fibrosis, and we're excited to offer additional help in making those stories come to life within the pages of our publication.

But first, please read a little about Melissa so you can familiarize yourself with her and her life experience as someone living 22 years post-lung transplant while also being a kidney recipient for 17 years! Plus, she is training to do a mini-triathlon for the first time in her life, is currently working full time as a school psychologist, is married to her best friend, and rescues animals. If you want to be published in *CF Roundtable*, reach out to us at: articles@usacfa.org.

Age: 41

Currently living in: Richmond, CA.

I have lived in the same house since I was five (yes, I still have my growth chart on the wall). It's a great place for gardening, hiking, and just lounging around. This was my parents' house, but I lost my father in 2004 and my mom in 2008. So it's been a great memory of the love and sacrifice they made to support a child with a chronic illness. Right now, my husband and I have two dogs, plus a roommate, and both his daughter and his dog, so there is never a dull moment.

When and where did you have your lung transplant?

I got my lung transplant at Stanford on November 9, 2001. I was at UCSF with pneumonia when I got the call and that was actually my third call. I arrived in an ambulance. The first call was on September 11, 2001; however, the organs were unable to be delivered because of the devastation in New York. I felt bad for being relieved the transplant didn't happen—even though I was

MELISSA TEEMAN

on the list, I felt I was not mentally prepared at the time. By the time I was transplanted, I was still terrified, but slightly more ready. While being wheeled into the room I asked if it was too late to turn around. The nurse said yes, and that was the scariest moment of my life. After the transplant, I woke up and could hear my parents talking to me, but I was unable to move my body. The next day, I was able to hold their hands and had a fairly stable recovery.

How long did you have dialysis? When and why did you have a kidney transplant?

I started dialysis in 2006. My kidney failed in part because I had back-to-back surgeries (sinus and wisdom teeth removal), but also because of the use of antibiotics and anti-rejection medication. My best friend's stepsister donated a kidney on December 3, 2010. She is a force to be reckoned with and has the absolute best taste in shoes. She is kind, thoughtful, and loves yoga.

When and how did you meet your husband?

My birthday is April 20th. My friends and I went out to celebrate at a dive bar on my birthday in 2007. My husband went to the same place with his friends. Two of our friends were dating at the time, so our group met with this group. His pickup line was, "So you're a teacher?" We really connected and called each other best friends forever from the first night we met. I told him about my CF, lung transplant, and that I was on dialysis. When I texted him two days later (ok, I was nervous—I was sure he'd never respond because of my health), he called and the story goes from there. He is an incredible man. He was by my side when I lost my mom, when I got my kidney; he encourages me every day and is always willing to go on a quick adventure. We've been together for 17 years, and just celebrated our 12th wedding anniversary on Sunday, August 20th, 2023.

Do you work? What do you do and do you like it?

I graduated from college in 2004. Although I wanted to be a school psychologist, I didn't want to be in school for the rest of my life and I was worried about transplant failure within five years, so I became a special education teacher. I did that for ten years until I got my kidney transplant. Since I knew I had a slim window, I went right back to college for school psychology and that is what I do today. I love my job, I am passionate

about my job, and my body tries to keep up. I am currently working full time—since COVID-19 I work from home three days a week and head into San Francisco twice a week. My work knows about my disability and they are extremely supportive and understanding. If I can't make it into work, my colleagues are more than understanding. It hasn't always been this way, but I find it easier to focus on my health and set boundaries—which is hard but critical—if I want to keep working.

Do you consider retiring at some point? What do you think you will do then with your time?

Right now, I am not sure if I am close to retiring. I love my job and it would break my heart to leave; however, with the increasing demands of my health, I know it's going to happen sooner than I want. My only goal is that I want to choose when to retire. I hope not to get so sick that I am forced out before I'm ready. I always want to decide and have control.

When I do finally retire, I'd love to work in some capacity, perhaps doing training or consulting work. I also love gardening and would love to start an urban gardening club for kids so they can learn how to make their own food and make healthy, cheap options as they grow up. I'd also like to travel, especially go back to Italy, Japan, and Costa Rica. My husband and I were previously active in animal rescue (dogs and cats). But after three foster failures (fostering dogs that we ended up keeping), we haven't been as involved. When I retire this is something I would love to do; walking rescue dogs, playing with cats, fostering, and transporting dogs from shelters to rescues, or from rescues to their new home.

You indicated you had sinus disease. What have you tried and what has helped you?

I mainly try to spice things up with my food (thank you chili powder and turmeric). I also try to chew my food thoroughly—it sounds weird but it really helps with my sinuses. I also do sinus massages, yoga, and I personally spend a lot of time with eucalyptus to help clean them out. Medically, my doctor put me on budesonide rinses for my sinuses, which has worked to decrease my polyps. However, I recommend talking to your doctor before adding anything to your sinuses!

What made you decide to start training for iron-man workouts or training in general?

My husband is an Iron Man! I am doing my first small triathlon this October; it's a 100-meter swim, a three-mile cycle, and one-mile run. My husband is my coach and I am most nervous about the swim. I am working to overcome my fear of being underwater.

Tell us about your CF camp experience as a child. What was that like?

Camp and generally being in the hospital were really fun as a child. In the hospital, we would have wheelchair water syringe races at night. We CFers would lie in one another's beds and watch movies. This is where I learned how to take my pills, gain weight, and just generally live as a child with CF. In the hospital, I would volunteer to help out the nurses with feeding and playing with young kids. When I was a teenager, my friends would come and we would play card games.

CF camp was the best week of my life. We stayed in cabins in the mountains, and everyone had to do their therapy in the morning. We all were trying to gain weight. But the best parts and memories were the activities—kayaking, swimming, archery, arts and crafts, holding snakes, and campfire skits. At camp I met Ana Modlin, and Ana and Isa Stenzel. I wish I had more specific memories, but, at this age, it's all filled with the activities we did, while the connections are a big blur.

What is the worst part of having CF for you currently?

The worst part about having CF for me currently is dealing with PTSD and just generally feeling "safe." As I describe it to my husband, it's like a bomb on my back just waiting to go off. The bomb being any physical or mental health problem that takes away the things I love. Physically, my GI requires the most management. I haven't gotten a blockage, but I do deal with bloating and constipation. I feel like when it comes to GI symptoms, I often don't eat because I get overwhelmed about what to eat, how it will affect my stomach, my diabetes, and finding a peaceful bathroom. Right now, I am trying to gain weight as a pescetarian.

Did you get diabetes post-transplant?

I did get diabetes post-transplant three years after my bilateral lung transplant). I take short-lasting insulin and use a Dexcom Sensor to help with controlling my blood sugars.

Why do you want to interview or help others write their CF stories for this publication?

As part of my job as a school psychologist, I write stories about the children I meet. I mainly do that through interviewing them, their family, and school staff. I love taking this information and helping parents and teachers experience the journey (both the highs and lows). I would love to be able to do this for CF patients. It can be hard to talk about our lives. This is one of the few interviews I have ever done, because I don't feel like anything is special or unique about me but being able to showcase someone is such an honor.

Did you ever consider having children?

Honestly, we didn't. We thought about fostering or adopting, but I knew I would have to give up my career, and it's something I have worked really hard for so, for now, it's just our dogs and doting on our nieces and nephews!

Tell us about your rescue pups.

I had three rescue dogs, as seen in my picture. Sadly, one has passed. Each

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who influenced you to be who you are today?

Yup, I have had many mentors. In high school, I had a few teachers who really inspired me to pursue my intellectual interests in STEM. In college, Dr. Michelle Monje and Dr. Erin Gibson inspired and cultivated my interests in medicine and biomedical research. Now, in medical school, Dr. Denise Chang, Dr. Jonathan Pan, and Dr. Ban Tsui are inspiring me to explore the field of anesthesiology and research within that field. And since I was a kid, my CF physicians have demonstrated to me what it means to be a great physician. ▲

If you would like to be interviewed for "In The Spotlight," please contact either Xan Nowakowski or Andrea Eisenman. Their contact information is on page 2.

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one is a little bit quirky. Cammie, our girl, broke her back so she needs to be lifted on the bed, and she will growl to let us know. She is quite a diva; she loves being sprayed with the hose, hiding under the blankets, snuggling, and eating seedless watermelon. Our other dog, Dennis, came to us scared of his shadow. He has learned how to play with dog toys, be good on a leash, and is always up to steal your snack or go for a walk. They have taught me how to read my energy (no curse words allowed in our house or the dogs will shake), give me daily exercise, and, most of all, learning to just be in the moment; it is all we really have. Naturally I am pretty structured because of my daily meds and treatments and the dogs seem to thrive with this.

What makes you laugh? What makes you cry?

Anything makes me laugh. I love laughing—silly animal stories, things kids say, smart and unexpected humor, to name a few. Thinking of the loss of my parents and how much sacrifice and love they gave me makes me cry. I only wish they were still here today. Kids and animals not having a voice and/or being given a crap shot at life also makes me cry, I guess in my case all animals are too much.

What is your mantra or saying that you live by?

I am grateful for each day; just the fact that I am still on this earth has me in utter awe!

My mom gave me a magnet after my lung transplant that said, "Never Never Give Up." So I try to remember that when things are hard. I also use the mantra "I am safe" when things are scary but manageable. Sometimes my body reacts to small problems as life threatening, so by saying that to myself, it can help a panic attack from continuing. ▲

If you would like to be connected with Melissa for help in sharing your story, send us an email at articles@usacfa.org!

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as molecular prosthetics for missing or dysfunctional CFTR protein channels, announced in June 2023 that the first healthy volunteer has been dosed with CM001 in a Phase 1 clinical trial. CM001 is a molecular prosthetic intended to treat people with cystic fibrosis (CF).This Novel approach could help treat the 10% of people with CF not eligible for CFTR modulator therapies.
https://tinyurl.com/3vk2me8t

Structural Changes In Lung Morphology Detected By MRI After Modulating Therapy With Elexacaftor/Tezacaftor/Ivacaftor In Adolescent And Adult Patients With Cystic Fibrosis

Elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) improves CFTR function in cystic fibrosis (CF) patients homozygous or heterozygous for F508del mutation. The aim of the study was to evaluate the response to ELX/TEZ/IVA treatment both clinically and morphologically in terms of bronchiectasis, bronchial wall thickening, mucus plugging, abscess and consolidations.

In patients with established cystic fibrosis disease the triple modulator ELX/TEZ/IVA improves CFTR function as demonstrated by better lung ventilation and nutritional status, reduced frequency of pulmonary exacerbations and decreased sweat chloride concentration.

Chest MRI detected significant favorable changes in lung morphology, particularly in mucus plugging and wall thickening/bronchiectasis. The study confirms the efficacy of ELX/TEZ/IVA in CF patients not only from a clinical point of view but also in terms of morphological changes of the lungs.
https://tinyurl.com/ykhnw9p7

Tissue-Specific Regulation Of CFTR Gene Expression

More than 2000 variations are described within the CFTR (Cystic Fibrosis Transmembrane Regulator) gene and related to large clinical issues from cystic fibrosis to mono-organ diseases. Although these CFTR-associated diseases have been well documented, a large phenotype spectrum is observed and correlations between phenotypes and genotypes are still not well estab-

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lished. Care for pwCF has largely improved these past years through the development of modulators (elexacaftor/tezacaftor/ivacaftor) treatment. Nonetheless, some patients are still not eligible or not responding. Moreover, some moderate CFTR disease cases remain misunderstood. These different findings highlight the necessity to understand the precise mechanism underlying the different patterns of disease's expression and the relationship between genotype and phenotype.
https://tinyurl.com/4ttd886c

CT Imaging Shows Specific Pancreatic Abnormalities In Persons With Cystic Fibrosis Related Diabetes

Cystic Fibrosis Related Diabetes (CFRD) is observed in 20-50% of patients with cystic fibrosis. Pancreas abnormalities on imaging, e.g. pancreas lipomatosis, are frequent in subjects with CF. The pancreas was classified on imaging according to 3 categories: normal, partial lipomatosis and complete lipomatosis of the pancreas. The presence or absence of pancreatic calcifications was also assessed. Forty-one CFRD and 53 CF control participants were included. Only 2% of the patients with CFRD had a normal pancreas, as compared with 30% of the participants from the CF control group. Lipomatosis was more frequent in subjects with CFRD and was related to exocrine pancreatic insufficiency (EPI) and to severe CFTR mutations (classes I to III). Nine participants with diabetes (22%) presented with pancreatic calcifications, versus none of the control participants. In conclusion, pancreas imaging was almost always abnormal in subjects with CFRD, while it was normal in a third of the CF control subjects. Pancreatic calcifications were specific to subjects with CFRD.
https://tinyurl.com/4u7ra4ab

Proteases and Antiproteases In Cystic

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Fibrosis: Pathogenetic Considerations And Therapeutic Strategies

The association between abnormal chloride transport, resulting from mutations in the cystic fibrosis transmembrane regulator (CFTR) gene, and the immunologic processes involved in the development of CF lung disease is poorly understood. However, neutrophil-dominated inflammation on the respiratory epithelial surface is a common finding in CF patients and suggests a mechanism for the immunologic abnormalities described in CF. Of particular importance for the pathophysiology of CF are proteases such as neutrophil elastase (NE) which are released from neutrophils in CF airways and cause direct structural damage to respiratory tissue. In CF, antiproteases are outnumbered by proteases and this protective mechanism is rendered ineffective. Restoration of this protease/antiprotease balance through antiprotease replacement therapy is currently under clinical investigation and preliminary results are promising.
https://tinyurl.com/5n9assv9

Clinical And Functional Efficacy Of Elexacaftor/Tezacaftor/Ivacaftor In People With Cystic Fibrosis Carrying The N1303K Mutation

Elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) significantly improves health outcomes in people with cystic fibrosis (pwCF) carrying one or two F508del mutations. According to in vitro assays performed in FRT cells, 178 additional mutations respond to ELX/TEZ/IVA. The N1303K mutation is not included in this list of mutations. Recent in vitro data suggested that ELX/TEZ/IVA increases N1303K-CFTR activity. Based on the in vitro response, eight patients commenced treatment with ELX/TEZ/IVA. This report supports the previously reported in vitro data, performed in human nasal and bronchial epithelial cells and intestinal organoids, that pwCF who carry the N1303K mutation have a significant clinical benefit by ELX/TEZ/IVA treatment.
https://tinyurl.com/2ekzwmyv

Combining SNAPs With Antibiotics Shows Enhanced Synergistic Efficacy Against *S. aureus and P. aeruginosa* Biofilms

Biofilm infections are associated with a high mortality risk for patients. Antibiotics perform poorly against biofilm communities, so high doses and prolonged treatments are often used in clinical settings. Researchers aimed to investigate the pairwise interactions of two synthetic nano-engineered antimicrobial polymers (SNAPs). The g-D50 copolymer was synergistic with penicillin and silver sulfadiazine against planktonic *Staphylococcus aureus* USA300 in synthetic

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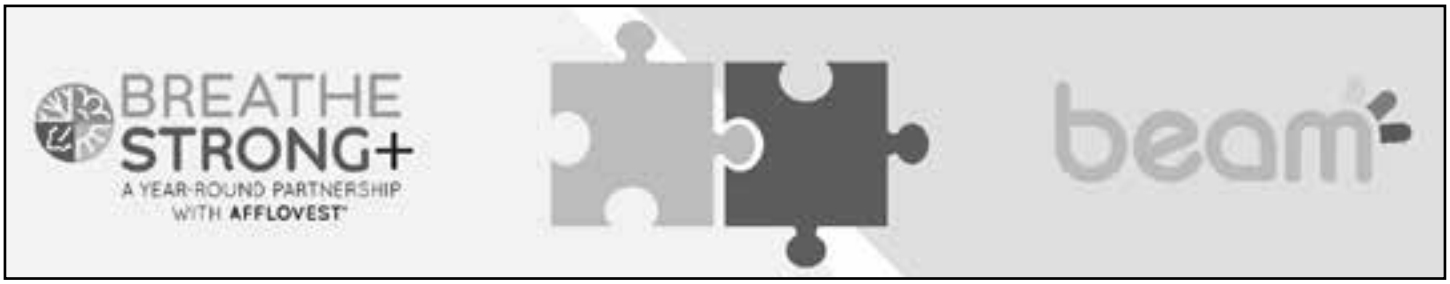

BreatheStrong+ Offers Free Access to Beam On-demand Classes

BreatheStrong+ is Beaming! Thanks to the generous support of the Helen M. Eisenman Fund, free access to adult Beam CF classes is now available exclusively through the BreatheStrong+ program through September 30, 2024. Together, BreatheStrong+, a program of Miles for Cystic Fibrosis, and Beam CF offer a free, comprehensive virtual wellness program to people with cystic fibrosis in the U.S. With the addition of Beam on-demand classes and watch parties, BreatheStrong+ now provides education, motivation, AND how-to resources to make good health a habit year-round.

BreatheStrong+ is free to people living with CF in the United States. This year-round, virtual wellness program focuses on fitness and nutrition as well as the impact wellness has on mental health. The program blends challenges, incentives and prizes, education, and motivation. The BreatheStrong+ community stays connected through fitness tracking as well as the scrapbook and chat features of the Charity Footprints app. Education, challenges and events can be found through social media, bi-weekly emails and chat.

Beam CF helps people living with cystic fibrosis through classes designed to improve physical and emotional well-being. Beam instructors are specialists working in or living with CF. Beam offers hundreds of on-demand classes to help improve strength and cardiovascular fitness, getting mucus from the lungs, building better posture and stronger bones, and so much more.

If you're puzzled by how to make healthy habits stick, join the BreatheStrong+ Beam community for all the pieces you need:

Education — BreatheStrong+ offers education on topics related to exercise, nutrition, mental health, and more. BreatheStrong+ Education Ambassadors understand what it's like to live with CF. These highly qualified CF fighters, family members and care providers contribute to the BreatheStrong+ blog, social channels and videos with vetted, quality content.

Motivation — Getting started and maintaining motivation can be a challenge for anyone on their wellness journey. The BreatheStrong+ online community is a place to share kudos and experiences, earn prizes and incentives, and virtually meet to encourage one another! Using the Charity Footprints app allows participants to connect a fitness tracker or manually enter activity to enter contests and visualize progress!

Expert Instruction — Beam instructors are experts in movement and have taken into consideration the challenges for all people living with CF. Whether someone is living with advanced CF lung disease, is on a modulator, has a port or PICC line, or is post-transplant, Beam sessions are designed to meet a variety of health needs. Best yet, they make it fun. Join one of the hundreds of available on-demand classes when it's convenient for you.

Well-being — Beam on-demand classes aren't just for your fitness journey! There are options that support every aspect of your well-being. You'll find classes featuring mindfulness, airway clearance, sleep and more!

BreatheStrong+ and Beam are ready to support people with CF at every stage of their lives. Kickstart your free membership or learn more at **BreatheStrongPlus.org**. ▲

Seeking Interviewees For Our In The Spotlight Column!

Would you like to be featured in our publication?
Do you want to be in the spotlight?
Let us shine a spotlight on you and your life!

We would love to showcase you and your life in our publication. If you are interested in being interviewed for an upcoming issue of *CF Roundtable*, please email us at CFRoundtable@usacfa.org. We will pair you with an interviewer who will arrange a time to talk and then write questions based on your answers for you to fill out at your leisure. Each interview is crafted to bring out what each person wants featured about themselves. To go along with your interview, we ask for two photos that will go into the publication: one headshot and one photo of you with your peeps, family, pet, on vacation, graduation, etc. We want you to shine so that others can benefit from your experiences living with this shared disease.

LECOINTRE continued from page 40

wound fluid. The combination of g-D50 and silver sulfadiazine showed a potent synergistic antibiofilm activity against *S. aureus* USA300 using in vitro and ex vivo wound biofilm models. The a-T50 copolymer was synergistic with colistin against planktonic *Pseudomonas aeruginosa* in synthetic cystic fibrosis medium, and this pair showed a potent synergistic antibiofilm activity against *P. aeruginosa* in an ex vivo cystic fibrosis lung model. SNAPs thus have the potential for increased antibiofilm performance in combination with certain antibiotics to shorten prolonged treatments and reduce dosages against biofilm infection.
https://tinyurl.com/ycks3523

Krystal Biotech Announces First Patient Dosed In Phase 1 Clinical Trial Of KB407 For The Treatment Of Cystic Fibrosis

In July 2023 first patient has been dosed at the Cystic Fibrosis Institute of Chicago in the Company's Phase 1 CORAL-1/US study evaluating KB407, an engineered HSV-1-based, aerosol-delivered, mutation agnostic, genetic medicine for the treatment of patients with cystic fibrosis (CF). The CORAL-1/US study is a multicenter, dose-escalation trial of KB407 in patients (n ~ 20) with CF regardless of their underlying genotype. Each administration of KB407 will be nebulized in under 30 minutes. This study will include three cohorts and enroll five subjects each in the first two cohorts and ten subjects in the last cohort. The primary endpoint of the trial will be the safety and tolerability of nebulized KB407. Changes in lung function from baseline will be assessed by the percent predicted forced expiratory volume in one second (ppFEV1). Vector shedding and biodistribution will also be assessed in blood, urine, buccal, and sputum samples. The CORAL-1/US study also includes a bronchoscopy sub-study for assessment of CFTR transgene expression in the airways at both the nucleic acid and protein levels. At select sites, subjects may undergo an optional bronchoscopy 24 to 96 hours after the last dose of KB407. The bronchoscopy will include bronchial brushings and endobronchial biopsies. KB407 is an investigational, redosable gene therapy designed to molecularly correct the underlying cause of CF by delivering two copies of the CFTR gene directly to the airways via nebulization.
https://tinyurl.com/2x95jveu

Role Of Hyperglycemia In Cystic Fibrosis Pulmonary Exacerbations.

Hyperglycemia could affect treatment response during cystic fibrosis (CF) exacerbations. Researchers aimed to evaluate the prevalence and associations of hyperglycemia with exacerbation outcomes. Feasibility of continuous glucose monitoring (CGM) during exacerbations was also evaluated. The STOP2 study assessed efficacy and safety of different durations of intravenous antibiotics for CF exacerbations. Hyperglycemia identified with random glucose is prevalent during CF exacerbations but not associated with changes in lung function or weight with exacerbation treatment. CGM is feasible and may provide a useful tool for hyperglycemia monitoring during exacerbations.
https://tinyurl.com/pvsw3a6k

Weight A Minute: Exploring The Effect On Weight And Body Composition After The Initiation Of

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Elexacaftor/Tezacaftor/Ivacaftor In Adults With CF

Life expectancy for people with CF continues to increase. However, a trend of overweight and obesity is emerging along with concern of developing comorbidities. Body composition (BC) is associated with several health indices. However, body mass index (BMI) does not provide information on BC. 109 PwCF (76 male) underwent assessments at both time points. In all PwCF a significant upward trend in BMI was observed. Males significantly gained more FFM compared to females, whilst prevalence of normal weight obesity increased primarily in females (25–38%). Routine BC assessment identifies individuals with elevated FM or depleted FFM enabling individualized care with the focus of optimizing BC.
https://tinyurl.com/45p9cn2d ▲

Aimee Lecointre is 38 and has CF. She lives in Salt Lake City, UT. She loves reading, cooking, writing, and spending time with her husband.

REMINDERS

- Please notify us immediately of any address changes. Returned mail wastes money and delays mailings.
- We would like to act as a referral source for active adult support groups. Please send us your group name, leader's name and phone number, number and age range of your members and geographical area covered, and we will add you to our referral list.
- Please let us know of the major occurrences in your life (e.g., marriages, births, completion of educational degrees or training, career advancement, transplants, anniversaries, birthdays), and we will print your information in **Milestones**.
- Share your ideas for **Focus Topics**, feature articles or any suggestions for improvements you may have to help make *CF Roundtable* more relevant and interesting to you.
- You can reach **USACFA** and **CF Roundtable** at any time by email at **cfroundtable@usacfa.org**
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- You may subscribe at www.cfroundtable.com

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IMPORTANT RESOURCES

Medical Assistance Tool (MAT): https://medicineassistancetool.org/ PhRMA's Medicine Assistance Tool (MAT) is a search engine designed to help patients, caregivers, and healthcare providers learn more about the resources available through the various biopharmaceutical industry programs. MAT is not its own patient assistance program, but rather a search engine for many of the patient assistance resources that the biopharmaceutical industry offers.

United Network for Organ Sharing (UNOS): Phone: 1-888-894-6361 http://www.unos.org/
Call for information on transplant centers, access for all patients needing organ transplants, and general transplant information.

Transplant Recipients International Organization, Inc. (TRIO): Phone: 1-800-TRIO-386 http://www.trioweb.org/index.shtml
An independent, nonprofit, international organization committed to improving the quality of life of transplant recipients and their families and the families of organ and tissue donors. For information, write to: TRIO, 7055 Heritage Hunt Dr, #307, Gainesville, VA 20155 or email them at: info@trioweb.org

American Organ Transplant Association (AOTA): Phone: 1-832-930-AOTA (2682) http://www.aotaonline.org/
Helps defray out-of-pocket travel expenses for transplant recipients. Helps to set up trust funds. For more information, write to: Administrative Service Center, American Organ Transplant Association, P. O. Box 418, Stilwell, KS 66085. Preferred method of contact is email: aotaonline@gmail.com

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