

Editor's Note: In October 2018, CFRI hosted an Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis, to bring the voices and experiences of those living with CF to representatives of the Food and Drug Administration. During this extremely impactful meeting, the painful realities of daily living with CF and the urgent need for new therapies were expressed. The summary Voice of the CF Patient Report can be read in its entirety at cfri.org. The following presentation by CF advocate Emily Kramer-Golinkoff provided moving testimony to the work still yet to be done.

Joy

By Emily Kramer-Golinkoff, MBE

Tuesday May 29th was a gorgeous day. Seventy degrees, blue sky, sun kissing my skin, one of those days where the universe was bursting with potential and it was hard not to feel content. I remember sitting outside the Perelman Center for Advanced Medicine when I got a call that changed everything. "Emily," my doctor said with uncharacteristic alarm, "Your x-ray revealed that your lung is partially collapsed in three places." I remember hanging up the phone, looking up at the big blue sky, and then back down at my body, stunned that a body so seemingly functional could be so secretly broken.

Meeting with my doctor, I learned that there were two treatment options for lung collapse, officially called pneumothorax, a life-threatening complication of advanced lung disease. The first was to closely monitor via x-ray and CT to see if time and rest could

resolve the collapse. The alternative was a procedure called pleurodesis that involved adhering the lung to the chest wall. The challenge is that it presents complications for transplant, an important consideration for someone with advanced stage disease like me. My hope was to do everything possible to avoid pleurodesis.

Ironically, treatment for lung collapse is antithetical to the standard of CF care in profound ways. Mainly no airway clearance and no exercise.

You can imagine the tailspin that throws your head into. The very strategies to which you devote four hours every single day, the ones that you depend on to control the infections ravaging your lungs, those are the very therapies that you must withhold so that your collapse might naturally heal. And therein lies the real predicament. It becomes a race. Can you go enough time without airway clearance for your lungs to re-inflate before an infection rages out of

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Emily Kramer-Golinkoff



Bacterial Community-Level Susceptibility Testing for CF Lung Infections

Lucas Hoffman, MD, PhD | University of Washington, Seattle

Chronic lung infections of people with cystic fibrosis (CF) do not respond to antibiotics as predicted by current concepts and culture methods. In general, antibiotics are chosen for treating CF patients based on the *in vitro* susceptibilities of a few isolates of the culturable "traditional pathogens" from their respiratory secretions. However, research demonstrates these methods do not result in the selection of antibiotics

that reliably lead to clinical improvement. In addition, researchers have been unable to establish significant, patient-level relationships between changes in standard CF respiratory culture results and clinical response to treatments. Therefore, the microbial determinants of clinical response to antibiotics are not clear, making it difficult to know how to improve our treatment approaches.

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CFRI Community

Spring 2019

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Letter from the Executive Director

The greatness of a community is most accurately measured by the compassionate actions of its members.

— Coretta Scott King

Dear Friends,

I hope this finds you well. While we continue to share the many stories and lessons learned from our Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis, now we are focused on bringing another Embrace Mothers Retreat, CF Summer Retreat for Adults with CF, National CF Education Conference, monthly podcasts, and several advocacy events to successful fruition. We are able to accomplish so much thanks to our wonderful organizational partners, our sponsors, and our generous – and compassionate – community members.

None of this is possible without you. Our research, advocacy, education, and support services are dependent upon the generosity of our community. Whether through the Mothers' Day campaign, the Jessica Fredrick Memorial CF Research Challenge Fund, car donations, or special events such as our new Move-a-Palooza, your participation is vital.

The sad truth is that we still have no cure. Our loved ones are still struggling to breathe. Cystic fibrosis remains a harsh and capricious disease, and each one of us plays a part in the search for a cure. In May 1995, I was told that my precious daughter Tess had cystic fibrosis. I will never forget the shock, sadness and fear I experienced upon receiving the news. Two months later, I was fortunate to attend a CFRI conference, where I found both information and community – my CF tribe.

I reflect daily on the many people who now enrich my life, with whom I initially shared only a CF connection. None of us would choose this path for our loved ones, but it is a relief to know that along with the extreme challenges and pain, there is also camaraderie, joy, friendship, shared purpose, and hope.

It is an honor to be a part of this great and compassionate community.

Warmly,



Siri Vaeth, MSW | CFRI Executive Director and Mother of an Adult Daughter with CF



News from the Board

Dear CFRI Community,

As always, the dedicated team at CFRI has been involved in diverse projects and activities to support, advocate for and engage our community. From participation in awareness events in Washington DC, to planning and implementing our educational programs, to raising funds for CF research, CFRI is adept at addressing our community's needs.

We remain in excellent fiscal shape, thanks to the generous support of members of our community and our corporate partners. We cannot accomplish our work without your commitment and partnership.

As I write this, members of the Research Advisory Committee (RAC) are vetting proposals submitted by CF researchers from across the country seeking funding through our Elizabeth Nash Memorial Fellowship and New Horizons Programs. The proposals describe innovative, high caliber cystic fibrosis research, and RAC members will have a challenging time narrowing the field. It is my



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cfri | Spring 2019

Summary: Voice of the Cystic Fibrosis Patient Report

While primarily known for causing progressive lung disease, cystic fibrosis (CF) leaves no organ system unscathed. It was with a sense of urgency that members of the CF community joined together at CFRI's Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis in October 2018, to share with Food and Drug Administration (FDA) representatives their experiences living with this complex disease, their perspectives on CF therapies, clinical trials and drug development, and their fervent hope that both drug developers and FDA regulators will intensify their efforts to advance more effective therapies for the disease.

The live-streamed meeting was part of FDA's Patient-Focused Drug Development Initiative, which seeks the patient perspective to provide context as regulatory decisions are made for new therapies, both during the drug development process and during the review of marketing applications for new drugs.

Key themes from the meeting are summarized below:

Cystic fibrosis has a devastating impact upon the lives of those living with the disease.

- Cystic fibrosis (CF) is a complex, capricious, multi-systemic, and debilitating disease.
- Respiratory complications were the primary challenge and source of concern for meeting participants, including lung infections, lung bleeds and lung collapse.
- Mental health issues and gastrointestinal complications also significantly impact CF patients' quality of life. Other common symptoms include CF-related diabetes, sinus polyps, liver disease, osteoporosis, and reproductive health challenges.
- Cystic fibrosis negatively impacts people's ability to spend time with friends and participate in social activities, as well as their attendance at work and/or school. Those living with CF worry deeply about advancing lung disease and death, as well as their ability to maintain financial stability and live independently.

Cystic fibrosis is a highly variable and heterogeneous disease, resulting in diagnosis, treatment and clinical trial design challenges.

- There are approximately 2,000 mutations of the CFTR gene that cause CF, with varying disease expression. For those with rare mutations, diagnosis may be challenging. Even when patients have the same mutation, disease symptoms and response to therapies can vary widely.
- Therapies for cystic fibrosis create a significant daily burden of care, with patients spending between 2 and 5 hours per day following their medical regimen, with hopes only of slowing disease progression. The new CFTR modulator therapies have improved lung function for some, but they are not a cure, and are not effective for all CFTR mutations.
- Side effects of medications often worsen health. Many drugs can damage the liver; some IV antibiotics lead to permanent deafness or trigger dangerous allergic responses; post-transplant immunosuppressants increase the risk of cancer and are



cfri Cystic Fibrosis Research Inc.

Voice of the Cystic Fibrosis Patient

A Summary Report of the Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis

Hosted by Cystic Fibrosis Research, Inc. (CFRI) — Held October 29, 2018

nephrotoxic; steroids used to reduce inflammation exacerbate CF-related diabetes; increasingly, pathogens develop resistance to available antibiotics.

- Individuals with CF who have received double lung transplants still must cope with the impacts of cystic fibrosis, along with an often debilitating regimen of immunosuppressants to hold off organ rejection.
- Clinical trials may be challenging due to the multiple mutations found among the CF population. Clinical trial exclusions that include lung function measures, age, specific mutations, BMI, and/or pathogens in the lungs may significantly limit the number of eligible patients for participation.
- Cystic fibrosis remains a progressive disease, and the vast majority of respondents noted that their symptoms had worsened over time.

Individuals with cystic fibrosis desire more effective treatments with reduced side effects and burden of care, and are committed to participation in the drug development process.

- The CF community has a strong desire for new therapies that reduce lung infections which lead to permanent lung function loss, pneumothorax, hemoptysis, hospitalizations, IV antibiotics, and lost work/school time. Individuals seek therapies that improve their ability to breathe. New drugs to address gastrointestinal complications must be developed. In light of the current treatment burden, new therapies that do not require additional time would be highly valued.

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Summary: Voice of the Cystic Fibrosis Patient Report *Continued from page 3*

- The CF community is known for its enthusiastic participation in clinical trials; the majority of meeting attendees were clinical trial participants – either currently or in the past.
- Meeting participants expressed strong willingness to assume risks in participation with clinical trials, also indicating that distance, placebo, blood draws, missed work, and multiple clinic visits would not discourage their participation.
- The desire for enhanced research, drug development and trials for members of the CF community with nonsense and other rare mutations was passionately expressed.
- Attendees expressed the desire to participate in clinical trials not only for one’s own personal health, but with the hope that others might benefit in the future.
- Individuals with cystic fibrosis have a strong desire to participate

in trials, and are often frustrated when they are excluded due to their FEV1. A consistent theme was a desire for drug developers and the FDA to consider expansion of criteria for CF clinical trials beyond the current FEV1 limitations to include number of exacerbations, quality of life, weight, etc.

- N-of-1 studies were encouraged due to the number of rare mutations in the CF community.

The Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis was a powerful event in illuminating the voices of those impacted by CF. It is hoped that this facilitates an ongoing conversation between the FDA and the CF patient community so as to inform those who assess the value and efficacy of CF-related therapies. Every day without new therapies matters.

The recording of the meeting is available on CFRI’s YouTube channel: <https://tinyurl.com/y77jt99h>.

To read the full report, go to cfri.org, and click on the “Advocacy” tab.

News from the Board

Continued from page 2

fervent wish that our funding continues to expand to keep pace with the requests for research grants.

In his cover article, CFRI-funded researcher Lucas Hoffman, MD, PhD, provides a wonderful overview of his inspired work. As a member of CFRI’s community, you are a part of this innovative

research. Thank you for your ongoing support. With your help we will continue to move closer to a cure, while enhancing the lives of those living with cystic fibrosis.

Peace and good health,



Bill Hult | Board President

Five Feet Apart: Bringing the CF Experience to the Big Screen

By Siri Vaeth, MSW

The film *Five Feet Apart* tells the story of Stella and Will, two teenagers with cystic fibrosis (CF) who meet and fall in love while in the hospital. Stella is listed for transplant, yet Will is ineligible due to culturing *Burkholderia cepacia*. The need to stay six feet apart to prevent cross infection is initially obvious, but as their relationship develops, the lines become blurred.

Five Feet Apart has triggered tremendous discussion in the CF community. Some celebrate the portrayal of CF on the big screen as an awareness-raising opportunity. Others question the message conveyed to young people with CF in terms of adherence to infection control protocols. We are a diverse community, and no film will accurately portray us in our full complexity.

CFRI was honored to host an online “town hall discussion” on the film, with over 950 people participating via Zoom and Facebook Live. As moderator, I was inspired by everyone’s honesty and candor in sharing what resonated most, including the relationships

with the medical care team, the isolation, the depression, and the intense frustration with the physical limits of infection control.

When asked to share their favorite scenes in the film, few mentioned Stella’s stealing a foot from CF. Rather, many people appreciated when Stella and Will disrobe and stand before each other, showing their scars, G-tubes and CF war wounds, exhibiting simultaneous vulnerability and intense power. This inspired a frank discussion about the body shame experienced by many with CF, and underscored the strength of that scene.

Other scenes were “favorites” due to their significance, including when Poe shares that his fear of being a burden on others has led him to reject their love. This scene triggered an extremely meaningful conversation, as



others expressed this same fear. Touch is a human need, and the scene in which Poe mourns that he cannot comfort Stella with a hug – a gesture taken for granted by those without CF – was painful and accurate.

Five Feet Apart was created with the input of Claire Wineland, and from the opening scene it is impossible not to think of her as we watch Stella posting her videos online. Claire is deeply missed, and it was wonderful to feel her presence throughout.

CFRI looks forward to hosting future “town hall” discussions. We are a community that hungers for connection, and we want to keep the conversation going.

Joy Continued from front cover

control? Lung collapse or infection, which is your poison?

And so on that beautiful day, I set off on my race against infection. My doctor sent me home on periodic high flow oxygen therapy to re-inflate my lung, my first time ever on supplemental oxygen, and he gave me strict instructions to come into the hospital for admission ASAP if I felt even a slight infection. After a lifetime of treatments, “time, rest, and no airway clearance” felt like an evil prescription of omission.

I knew enough about lung collapse to know that it was the next step to end-stage disease and I felt desperate for information. I plunged into the online CF community. I read story after story of people who experienced lung collapses and permanently lost huge chunks of lung function, who were unexpectedly thrust into the terrifying world of transplant, or even worse, for whom the treatment for lung collapse questioned their future of transplant altogether.

I read of people who had pleurodesis for whom it proved unsuccessful; people who told me they were never the same again, for whom that first lung collapse was just the start of many more to come. And I read, time and time again, that the pain of pleurodesis was utterly excruciating.

I’m a resilient, hopeful person. All I was looking for was the tiniest glimmer of hope, of possibility that it could be okay. But the deeper I probed, all I found were stories of cat-

astrophe or, at best, slow demise. I couldn’t find even one story with a positive ending.

The more I learned, the longer the list of restrictions grew. No airway clearance, no exercise, no spirometry, no lifting. And as horrific as they were, I felt I could endure. A lifetime with a disease with a penchant for rearing its head at inopportune times had taught me how to get brutally knocked down and stand back up again. And yet, the one thing that made me question whether or not I could endure was the one about joy.

Learning that flying was off limits not only immediately following lung collapse but that it would forever be a major trigger, that was the one that threatened to crush my soul. Even the most unimaginable pain and suffering are endurable with meaningful punctuations of joy. For me, that joy is traveling to faraway places. The thought that this disease could not only rob my function and my future, but that it could also take my single greatest joy - that was the part that wrecked my heart, that made me question, for the first time in my life, if I could indeed endure.

CF, you take my breath, you take my friends, you take my dreams of a career and a family. You take my hope, my promise, my very potential. You take my future, and now, you take my joy.

Emily co-founded Emily’s Entourage, a 501(c)3 that accelerates research and drug development for nonsense mutations of CF. To learn more, go to emilysentourage.org, where you will also find their social media links.



Emily Kramer-Golinkoff with her mother – and Emily’s Entourage cofounder – Liza Kamer, MSW.

Move-a-Palooza!

September 14 - 21, 2019

Moving toward a cure!

Join Your Peers Nationwide:
Raise Cystic Fibrosis Awareness
& Support the Search for a Cure!



Pick your favorite way to move: reps in a gym, running, hula hooping, swimming – you decide! Ask your friends and family to sponsor you. CFRI will provide a link to our online fundraising platform, where your supporters can make donations. Choose your day - anytime between September 14 and September 21. Take pictures and/or video and post on social media #CFRI-Moveapalooza. CFRI suggests a \$100 minimum goal per participant, but there’s no required amount, and no penalty if you don’t reach your goal. Those who raise a minimum of \$100 will receive a Move-a-Palooza t-shirt!

Questions? Email Tony Adessa at tadessa@cfri.org or call 650.665-7586.

CF Quality of Life Programs: Supporting the Mental Health of Our Community

It is no surprise that those impacted by cystic fibrosis (CF) have higher rates of depression and anxiety. With its unpredictability, daily treatment burden, and diverse symptoms, cystic fibrosis is a challenging disease for those diagnosed, as well as for those who love them. Making the issue more complicated, studies show that depression can negatively impact adherence to one's medical regimen. In response, CFRI offers a range of programs to address the psychosocial needs of our community.

- **Counseling Support:** CFRI provides up to \$120 per session for six sessions of counseling to individuals with CF (children and adults), their parents, partners, spouses and siblings with the licensed provider of their choice. Participants must live in the U.S.
- **Caregivers Support Groups:** Two groups are offered – one for parents of children with CF, and another for parents/spouses/partners of adults with CF – and facilitated by Meg Dvorak, LCSW, CF social worker. The groups are held in person and via Zoom, and caregivers participate from across the country.
- **“Living Mindfully with CF” Online Classes:** Drawing upon Mindfulness Based Stress Reduction techniques and focusing these practices to living with CF, this class helps to reduce anxiety and depression and is offered to individuals with CF and their family members. Taught by Julie Desch, MD, who herself lives with CF.

These programs are offered at no charge to our community members, thanks to our supportive sponsors. For more information, visit our website, or email Sabine Brants at sbrants@cfri.org.

Partners in Living Initiative – CF Quality of Life Programs are supported through grants from Vertex Pharmaceuticals, Gilead Sciences, Genentech, Chiesi USA, Horizon Pharma, and contributions through CFRI's CF Quality of Life Program, a Living Legacy of Peter and Kathy Judge.



Jessica Fredrick Memorial CF Research Challenge Circle and Fund

Real generosity toward the future lies in giving all to the present... — Albert Camus

CFRI's Jessica Fredrick Memorial CF Research Challenge Circle gives generously to inspire others to join the search for new CF therapies and a cure. Last year, members of our circle contributed \$90,000 so as to match – dollar for dollar – donations from individuals committed to CF research. Together, these donations funded \$180,000 in our CF Research Challenge Fund, which will be used for our New Horizon and Elizabeth Nash Memorial Fellowship CF research awards.

Please join this inspiring group! Become a member of the 2019 Jessica Fredrick Memorial CF Research Challenge Circle by making a minimum gift of

\$2,500. You will help inspire others to make the dream of a CF cure a reality.

Our Circle is named in memory of Jessica Fredrick, pictured right, who lost her battle with CF at the age of 21. Despite tremendous progress in CF therapies, we continue to lose our loved ones to this cruel disease, and there is still no cure. We need your help to improve and save the lives of our loved ones.

Please give today. If you are unable to join the Circle, please consider making a gift to the Research Challenge Fund, which will be designated for CF research awards. By giving all to the present, you are generously supporting the future hopes of those with CF.



Tributes

Our “In Memory of” and “In Honor of” pages provide the opportunity to honor a person, or family, or to remember a loved one. If you want your donation to honor or remember someone special, please include the person's name and address with your donation.

At your request, we will send an acknowledgment of your gift to the person you designate.

Please mail your contributions to:

CFRI
1731 Embarcadero Road,
Suite 210, Palo Alto, CA 94303

In Honor of

September 1, 2018 — March 15, 2019

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Sadie Anderson
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Lisa Avery
Haleigh Baker
Kyle Baker
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Lucy, Charlie and Dylan Barnes Markell
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In Memory of

September 1, 2018 — March 15, 2019

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Renee, Murry, and Bob Wolfson
Josiah Woodhouse
Betty Woods

Bacterial Community-Level Susceptibility Testing for CF Lung Infections

Continued from front cover

A growing body of research has identified candidate explanations for this disconnect between *in vitro* test results and patient responses to antibiotics. Specifically, evidence suggests that the targeted traditional pathogens, such as *Pseudomonas aeruginosa* and *Staphylococcus aureus*, infect diverse populations of CF patients who have many characteristics, including antibiotic susceptibilities, that are not reflected by the few isolates that are currently identified and tested. In addition, culture-independent (microbiome) CF respiratory studies have detected many microbial species not identified by current clinical laboratory methods as commonly co-infecting CF patients' airways. As such, the microbes causing CF lung infections are very diverse—in terms of both numbers of species and variants of each species—and this diversity is not reflected by routine clinical laboratory practices, likely having substantial impact on both microbiological and clinical responses to treatments such as antibiotics. Therefore, improved methods for selecting antibiotics should reflect the entire, diverse infecting microbiota.

We have developed a novel method for analyzing the aggregate antibiotic responses of the complete microbial communities that

infect CF airways. This method, which we call *community-level susceptibility testing* (or CLST), uses sputum from CF patients to inoculate arrays of different antibiotics that are incubated under aerobic, microaerobic, and anaerobic conditions, all of which occur in patient lungs. The effects of the added antibiotics are measured by monitoring collective community growth over time. Therefore, CLST measures the response of the entire infecting microbial community, rather than just a few cultured bacterial isolates, to candidate antibiotic treatments, under different conditions relevant for the CF airway. The resulting test conditions can be sampled and analyzed both by culture and sequencing to more accurately quantify the *in vitro* microbial community responses to specific antibiotics. This method therefore allows more comprehensive and precise measurement of microbiological responses to antibiotics and other chemicals *in vitro* in a polymicrobial context than do traditional culture methods.

With CFRI support, we are using CLST coupled with both culture and next-generation sequencing to analyze archived sputum samples from 30 children and adults with CF collected before, during, and after a month-long treatment with the common CF antibiotic treatment inhaled tobramycin, and to compare CLST outcomes with both

clinical and microbiological measures of patient response to this treatment. Microbiological outcomes include quantitative cultures of each pre- and post-treatment sputum sample for traditional pathogens, including *P. aeruginosa* and *S. aureus*.



Lucas Hoffman, MD, PhD

With these techniques and resources, we are testing the hypothesis that the effects of tobramycin on complete CF sputum microbial communities *in vitro* using CLST will better reflect the clinical and sputum microbiological changes of patients with inhaled tobramycin treatment than will either of two standard laboratory measures: *in vitro* tobramycin susceptibilities of traditional pathogen sputum isolates, or changes in sputum abundances of those traditional pathogens. We look forward to sharing the results of our research as it progresses.

Dr. Hoffman will be presenting an update on his research at CFRI's National CF Education Conference, which will be held July 26 – 28.

2019 Mothers' Day Fundraiser Brings Awareness and Hope!



It is an annual tradition that involves hundreds of people, promotes awareness of cystic fibrosis (CF) among our friends and family around the globe, and ultimately raises nearly \$110,000 to support CFRI's vital services to the CF community. Our Mothers' Day fundraiser has long been a success thanks to the participation of our incredible community.

Contact us at CFRI, and we will provide you with everything you need, including the beautiful invitation pictured here, created by Rachel Reveles in honor of her sister Megan, who lives with CF. Mail the invitations to your family and friends, encouraging them to reflect upon their loved ones with cystic fibrosis (CF) and to support CFRI's outstanding research, education and support programs that improve the lives of people with cystic fibrosis.

Prefer to send invitations online? We've got you covered. Go to cfri.org/mothersday. Is Facebook easier for you? We can help you to easily make that happen. Please call our office at 650.665.7559, email cfri@cfri.org, or sign-up online at www.cfri.org.

From CF Fitness to CFRI-Funded Research: CF Community Voices Has It All!

By the community and for the community, CFRI's CF Community Voices was created to share information and insights about a wide variety of topics. Recent releases include podcasts on innovative CF research, complementary therapies, and a recording of an online "Town Hall" discussion on the film *Five Feet Apart*. New episodes are released monthly, and are available on CFRI's podhosting site: cfri.podbean.com. You can watch or listen at the site, or download to enjoy in the car or while exercising. Episodes are also posted on CFRI's YouTube channel. We look forward to sharing our community's diverse voices. Generously sponsored by Vertex Pharmaceuticals, Chiesi USA and Gilead Sciences

Embrace: A Retreat for Mothers of Those with CF

The fifth annual retreat for mothers of children and adults diagnosed with cystic fibrosis (CF) will be held May 3rd – May 5th at Vallombrosa Center in Menlo Park, California, where women can learn about the latest resources for navigating the CF road, while participating in therapeutic art projects, yoga, journaling, support groups, and needed respite time. Studies demonstrate that mothers of children with CF

have extremely high rates of depression and anxiety, and that this can impact their children's adherence to their medical regimen. Women are travelling from throughout the United States to attend this powerful event, which sold out two months in advance. The retreat provides creative strategies for coping with the challenges of the CF journey while creating a lasting support network.



Sponsored by AbbVie and Gilead Sciences.

2019 CF Summer Retreat: A Place of Learning, Support and Community for Those with CF July 20 – 26, 2019 / Vallombrosa Retreat Center, Menlo Park, CA

Are you an adult with CF feeling isolated from your CF peers? CFRI's CF Summer Retreat provides a safe and welcoming environment for adults with cystic fibrosis. It was created to enhance education, positive coping skills, and social support for people

who share common experiences with CF and are increasingly isolated due to cross infection protocols.

The retreat provides health-related and psychosocial support programs and activities including:

- Daily exercise activities tailored to individuals' unique capacities;
- Educational workshops with guest speakers from local CF centers, addressing such issues as transplant, gastrointestinal challenges, hemoptysis, nutrition, and sinus disease;
- Daily rap sessions (support groups) to support positive mental health;
- Special events and dedicated time to connect, network, and socialize with others.

Attendees enthusiastically report that the retreat provides new information about CF therapies and treatments, dramatically improves psychosocial health, and provides resources and strategies for coping with the daily challenges of CF. The retreat is open to adults with CF (in adherence with our Cross-Infection policy*) as well as their adult family members and supportive peers. Join us! For more information, go to www.cfri.org or call Mary Convento at 650.665.7559.

** Retreat attendees with CF must have a sputum culture prior to attending, and a medical release form signed by one's physician attesting to the absence of specific pathogens. CFRI's infection policy can be found on our website.*

CF Summer Retreat is generously sponsored by Gilead Sciences and AbbVie.



A Brief Reminder To Sterilize Your Nebulizers

The importance of clean and sterile nebulizers for preventing infections in the lungs of those with cystic fibrosis (CF) cannot be overstated. Those with CF typically use some form of airway clearance technique several times per day along with nebulized medications. The most common nebulizers are reusable, and if not cleaned properly are liable to harbor infectious pathogens.

The sterilization of nebulizers is absolutely essential for infection control. Between every treatment, nebulizers must be cleaned, disinfected, rinsed with sterile water, and air-dried. Special attention must be given to the disinfection step, and there are both hot and cold methods to achieve nebulizer disinfection.

Cold method options involve soaking the nebulizer in any of the following solutions:

- Bleach solution for 3 minutes (1 part bleach to 50 parts water)
- 70% Isopropyl alcohol for 5 minutes
- 3% Hydrogen peroxide for 30 minutes.

Hot disinfection options:

- Boil the nebulizer for 5 minutes
- Microwave the nebulizer in water on high for 5 minutes
- Wash in a dishwasher for 30 minutes (water must be greater than 158 degrees Fahrenheit)
- Treat with an electric steam sterilizer (sold in baby supply stores to sterilize bottles)

The cleaning and disinfection of respiratory care equipment is an essential strategy for infection prevention and optimal health. Always check with the particular nebulizer manufacturer for the method they recommend.



CFRI Research Awards Program Supports Promising Cystic Fibrosis Research Nationwide

Through New Horizons Research Campaign and Elizabeth Nash Memorial Fellowship

New Horizons

Through the New Horizons grants, CFRI funds research of the highest scientific quality in academic and hospital institutions. The program provides a minimum of two \$70,000 awards per year for two years to Principal Investigators (PIs). Funded projects are highly relevant to CF and are original, probing and/or pioneering a new approach to a therapy or cure. The NH awards also provide seed funding for promising new lines of basic and clinical CF research that show prospects of future funding by other sources, such as the National Institutes of Health (NIH).

Current New Horizons Researchers include:

- Jeff Wine, PhD, and Nam Soo Joo, PhD: A Novel Approach to Improve Mucociliary Clearance for CF Patients; *Co-Principal Investigators, Stanford University*
- Paul Quinton, PhD, and Guillermo Flores-Delgado, PhD: Assessing the Role of Secretory and Absorptive Epithelium Lining Conductive Airways; *Co-Principal Investigators, University of California San Diego*
- AKM Shamsuddin, PhD: Pharmacology of CFTR HCO₃ Secretion in Native Small Airways in Health and Disease; *Principal Investigator, University of California San Diego*
- Martina Gentsch, PhD: Physiological Models of Cystic Fibrosis Airway Cultures that Mimic the In Vivo Environment for Therapeutic Testing; *Principal Investigator, University of North Carolina at Chapel Hill*

- Lucas Hoffman, MD, PhD: Bacterial Community-Level Susceptibility Testing for CF Lung Infections; *Principal Investigator, University of Washington, Seattle and Seattle Children's Hospital*
- Elizabeth Kramer, MD, PhD: The Role of TGF in Driving Airway Hyperresponsiveness and Smooth Muscle Dysfunction in Cystic Fibrosis; *Principal Investigator, Cincinnati Children's Hospital Medical Center*

Elizabeth Nash Memorial Fellowship

The Elizabeth Nash Memorial Fellowship (ENMF) provides annual funding to Post-Doctoral Fellows engaged in original, probing and/or pioneering CF-related research at academic and hospital institutions nation-wide and encourages collaborative research and communication between the institutions and Principal Investigators. The fellowship is named in memory of geneticist Elizabeth Nash, who had CF and served as CFRI's RAC Chairperson. Up to \$60,000 per year for two years is awarded to a research institution to support a principal investigator, who in turn appoints a postdoctoral research fellow.

Current Elizabeth Nash Memorial Fellowship Researchers include:

- Kenichi Okuda, MD: Localization of CFTR and Secretory Mucins MUC5AC and MUC5B in Human Airway; *Postdoctoral Fellow, University of North Carolina Chapel Hill (Richard Boucher, MD, Principal Investigator)*
- Cynthia B. Silveira: Bacteriophage-Mediated Spread of Virulence Factors in Cystic Fibrosis Microbiomes; *Postdoctoral Fellow, San Diego State University (Forest Rowher, PhD, Principal Investigator)*



CFRI Is Your Partner for Life

Together We Bring Research & Programs to Life

- **HOLD YOUR OWN EVENT:** Cocktails for a cure, yard sales – no idea is too big or too small. Think “out of the box,” and we'll help you make it happen.
- **FACEBOOK:** Many community members create fundraisers for CFRI by donating their birthdays on Facebook. Go to <https://www.facebook.com/cfri.org/>, scroll down to Fundraisers, and click on Create!
- **MOVE-A-PALOOZA:** Our inaugural event! You choose the activity (walk, swim, pogo stick, burpees), get a team together, collect pledges, and move! September 14 – 21, 2019.
- **MONTHLY GIVING:** *Champions of Hope!* Donations to Champions of Hope provide a revenue stream to support research to find a cure for CF and enhance CFRI's programs in CF education, support and advocacy. To participate, go to our website or contact Tony Adessa (see below).
- **TRIBUTES:** “In Honor Of” and “In Memory Of” – Recognize a loved one with your choice of gift. CFRI will promptly send an acknowledgement letter to your designee.
- **STOCK DONATIONS TO CFRI:** Donating appreciated stock avoids capital gains taxes incurred had the stock been sold. You're also entitled to an income tax charitable deduction for the stock gift date's fair market value.
- **PLANNED GIVING:** offers benefits that can include increased income, substantial tax savings, opportunity to meet your philanthropic goals, and the satisfaction of making a very significant gift to CFRI during your lifetime.
- **BEQUESTS:** Include CFRI as a beneficiary in your Will or Living Trust. At the time of your passing, your designated amount would come to CFRI – tax-free to your heirs and CFRI.

For more information, please contact Tony Adessa, CFRI's Development Manager: 650.665.7586 or tadessa@cfri.org.

CFRI's Moonlight Masquerade

A Gala to Support CF Research, Education, Support & Advocacy Programs



Save the date! CFRI's Moonlight Masquerade will be held Saturday, October 26, 2019 from 6:00 pm to 11:00 pm at the beautiful and historic Hillsborough Racquet Club in Hillsborough, California. Enjoy gourmet mardi gras delicacies, fine wines and fabulous entertainment while celebrating progress in the field of CF research and therapies. Honor our 2019 CF Champion, an individual dedicated to improving the lives of those with cystic fibrosis. Bid in our exciting auction, and end the night on the dance floor. All proceeds are directed to CFRI's research, education, advocacy, and support programs to improve the lives of those with CF.

Cost: \$150 per person. Sponsorship packages are available. For more information go to www.cfri.org, or call **650.665.7586**. Generously sponsored to date by Vertex Pharmaceuticals, AbbVie, Chiesi USA, and NBC Bay Area.

Many Voices ~ One Voice: We Are Stronger Every Day By Siri Vaeth, MSW

Cystic fibrosis (CF) is an isolating disease due to the small number of individuals diagnosed in the U.S., as well as the cross-infection risks that keep our community apart. CFRI's Many Voices ~ One Voice Cystic Fibrosis Advocacy and Awareness Program brings people together for a shared purpose, providing opportunities for people to collectively affect change at a state and federal level. By heightening awareness of issues impacting our community and providing easy tools to implement calls to action, the campaign empowers those impacted by CF to express their opinions to those in positions of influence. Many Voices ~ One Voice has inspired a new wave of engaged CF advocates whose impact will endure into the future.

This year we continue to educate the general public, Food and Drug Administration representatives, and legislators about the burdens and complications of the disease, lack of cure, and the impact of its "rare disease" status upon those diagnosed. We track Drug Utilization Review Board decisions in states across the country and advocate on behalf of the CF community when decisions negatively impact access to CF-related therapies. Using email, print and social media to drive online calls to action, thousands of emails have been sent to elected representatives, encouraging their consideration of our community's needs.



We seek to build and strengthen coalitions of CF and rare disease agencies and activists to increase state and national legislative awareness of the disease, and work closely with our organizational partners in the Cystic Fibrosis Engagement Network. In addition, CFRI closely follows and takes positions on national and state legislation important to individuals with CF, such as access to treatments, the need for basic science CF research and continued drug research for next generation therapies.

Most significantly, CFRI seeks to increase our base of dedicated activists and donors. Our impact depends on the participation of our community. I invite you to join CFRI in raising CF awareness and advocating for research funding, new drug development, and access to therapies and quality care. Go to cfri.org and click on the Advocacy tab. Our many voices combined as one will ensure that our community's needs are heard.

SAVE THE DATES!

Please sign up to receive our weekly eNewsletter to stay informed of our many programs and events!

CF Caregivers Support Groups

Third Tuesday of Every Month

May 21 • June 18 • July 16

August 20 • September 17

October 15

5:00 pm – 6:00 pm PST:

Go to www.cfri.org for information.

Participate in person or by phone

Sponsored by Vertex Pharmaceuticals, Horizon Pharma, Gilead Sciences, and Chiesi USA

Embrace Mothers' Retreat

May 3 – May 5, 2019

Vallombrosa Center, Menlo Park, CA

Sponsored by Gilead Sciences and AbbVie

CF Summer Retreat

July 20 – July 26, 2019

Vallombrosa Center, Menlo Park, CA

Sponsored by AbbVie and Gilead Sciences

CFRI's 32nd National CF Education Conference

July 26 – July 28, 2019

Pullman San Francisco Bay
Redwood City, CA

(See back page for schedule and sponsors)

CFRI's Move-a-Palooza!

September 14 – September 21, 2019

However you want to move &
Wherever you want to move!

CFRI's Moonlight Masquerade

October 26, 2019

Hillsborough Racquet Club
Hillsborough, CA

Sponsored by Vertex Pharmaceuticals, NBC Bay Area, AbbVie and Chiesi USA

For information or to register for these events, please email cfri@cfri.org or call 650.665.7559.



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CFRI's mailing list is confidential. We do not sell our list, nor do we give out any names or addresses under any circumstance.

CFRI Mission

Cystic Fibrosis Research, Inc. exists to fund research, to provide education and personal support, and to spread awareness of cystic fibrosis, a life-threatening genetic disease.

CFRI Vision

As we work to find a cure for cystic fibrosis, CFRI envisions informing, engaging and empowering the CF community to help all who have this challenging disease attain the highest possible quality of life.

For their generous support of **CFRI Community**, special thanks to:

Vertex Pharmaceuticals, Genentech, Gilead Sciences, AbbVie, Chiesi USA, Proteostasis Therapeutics, and Translate Bio

Visit our website at:
www.cfri.org

for more information about us and about cystic fibrosis.

Call toll free: **855.cfri.now**

CFRI's 32nd National Cystic Fibrosis Education Conference: *Charting the CF Course*

July 26 – July 28, 2019 / Pullman San Francisco Bay – Redwood City, CA

Our 32nd annual conference promises to be our most exciting thus far, offering outstanding speakers, receptions, support groups, workshops, and the opportunity to connect with your CF community from across the country.

Join us!

Speakers Include:

- Jerry Cahill
- Travis Flores
- Mary Abigail Garcia, MD
- Nadia Islam, PhD
- Traci M. Kazmerski, MD, MS
- David Nichols, MD
- Steven M. Rowe, MD, MSPH
- Emily Schaller

CFRI-Funded Researchers:

Presentations on Saturday/Sunday by Researchers from Stanford, UC San Diego, University of Washington, Cincinnati Children's Medical Center, UNC Chapel Hill, and San Diego State University.

For those attending only the Research Track, please go to our website for details on special pricing.

Generously sponsored by: **Vertex Pharmaceuticals, Genentech, Gilead Sciences, Chiesi USA, AbbVie, Ionis Pharmaceuticals** and the **Boomer Esiason Foundation**

Cystic Fibrosis Research, Inc. a 501(c)(3) nonprofit organization Federal EIN# 51-0169988



Early Bird Registration: for the full conference (on or before 7/16/19) **\$185 per person**

Regular Registration: (7/17/19 and after) **\$215 per person**
Registration includes conference meals, reference materials, presentations, receptions, and support groups.

To ensure good health for all, please use proper hygiene practices. All participants/guests with CF must adhere to CFRI's Infection Control Guidelines. See **www.cfri.org** for specifics.

For more information, visit **www.cfri.org** or call **855.cfri.now**.

