

CFRI's 33rd National Cystic Fibrosis Education Conference: Speaker Abstracts

At CFRI's 33rd National CF Education Conference, *Partners in Progress*, held virtually August 1 and 2, 2020, nationally-renowned speakers from across the country shared their expertise and experience on a wide range of CF-related topics. These presentations are now available for viewing on CFRI's YouTube channel. The abstracts below are followed by a link to each specific presentation.

Double Jeopardy: CF in the Age of COVID-19

Richard Moss, MD
Stanford University, Palo Alto, CA

This talk reviews traits of COVID-19, such as how the virus infects tissues and the immune-inflammatory response, primary symptoms and treatments, risk factors, and prospects for protection by natural infection or vaccination. Reports show that the CF community has done very well in using public health measures of physical distancing, face coverings and hand hygiene to keep infection rates low. This is attested to by infection rates of 3 to 4.6% of those CF patients tested being positive for the virus.



Moreover, it appears that most CF patients who do become infected have had generally favorable outcomes with only 5 deaths as of 25 June 2020 reported among 151 known cases, a case fatality rate (CFR) of 3.3%,

which is lower than the current CFR in the USA (5.0%) or worldwide (5.1%). Within CF as a main diagnosis, additional risk factors that seem to emerge for COVID-19 illness in

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A Multi-'Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis

— Kehoon Lee, PhD, *The Pathogen and Microbiome Institute; Northern Arizona University*

The human microbiota is composed of trillions of microorganisms (bacteria, fungi, archaea, and viruses) that play a myriad of roles in human health and disease. There are approximately 1 to 1.2x more microbial cells living in and on the human host and, impressively, these microbes encode approximately 100x more genes than the human genome. Recent studies, typically

focused on the gut microbiota, have highlighted the various roles our resident microbes play in digestion of complex carbohydrates, education of the immune system, and even contribute to health in peripheral organs such as the lungs and brain.

Despite these advances in our understanding of the role of the gut microbes in health

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CFRI Community

Fall 2020

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Letter from the Executive Director

Institute: a society or organization having a particular object or common factor, especially a scientific, educational, or social one.

Dear Friends,

I hope this finds you safe and well. All of us at CFRI are thinking of those who have lost loved ones to COVID-19, and those whose lives have been upturned due to wildfires, tornados and hurricanes. We are a resilient community, and this year is a testament to our strength.

2020 marks another kind of milestone as CFRI celebrates 45 years of research funding and service to our community. CFRI was formed by a group of parents whose children with CF were not expected to survive their teen years. With your support, we have grown tremendously since 1975, still funding innovative CF research while incorporating education, psychosocial support and advocacy into our mission. We are truly a CF scientific, educational and social institute.

CF remains a harsh and capricious disease. Many members of our community are still waiting for transformative therapies. Each one of us plays a part in the search for a cure.

As we approach the end of 2020, let us reaffirm our collective commitment to the work that remains to be done. Thank you for your consistent generosity and support.

Warmly,



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



News from the Board

Dear CFRI Community,

I hope that you are safe and well. As COVID-19 continues to impact nearly every aspect of our lives, I am proud of CFRI's response in supporting our community. As predicted, the pandemic has had an impact on CFRI's finances. The Board of Directors and staff have been very creative in seeking new sources of revenue while reducing expenses. Thanks to their dedication, we have maintained all levels of services with a balanced budget. Our recent audit once again confirms that we are a financially stable organization with excellent fiscal management.

The funding of cutting-edge research remains key. We are in the process of evaluating letters of intent for our next round of Elizabeth Nash Memorial Fellowship and New Horizons Program awards. 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 | President, CFRI Board of Directors



the reported cases include age, CF-related diabetes and lung transplantation, which are also known risk factors in the general population; but advanced lung disease is not over-represented. Going forward, there are many important issues for the CF community vis á vis COVID-19, including maintaining overall and CF Center medical care and status measures, access to CF medications, status of clinical research and drug trials, avoiding discrimination if ICUs become overwhelmed during spikes, and how CF will fit in a predictable queue for vaccination both during clinical trials and once one or more vaccines become licensed and available. <https://tinyurl.com/y5955ovp>



Richard Moss, MD

The Potential of Emerging Technologies for The Treatment of Cystic Fibrosis

Marie Egan, MD
Yale School of Medicine

Although effective CFTR modulator therapy has the potential to change the lives of many patients with cystic fibrosis (CF), it is unlikely that these drugs will be a game



Marie Egan, MD

changing therapy for all. There are about 10% of patients with CF who don't produce a mutant protein to modulate, potentiate or optimize, and for these patients, alternative approaches may be needed. There is a need to develop new therapeutic approaches that can work for this patient population and can advance CF therapies. These new therapies will include nucleic acid and genetic-based therapies and each approach will result in functional CFTR protein in previously affected CF cells. This talk examines the potential of RNA therapies, gene transfer therapies and gene editing therapies for the treat-

ment of CF, as well as the challenges that will need to be faced as we harness the power of these emerging therapies. <https://tinyurl.com/y5c4ahk4>

Anti-Inflammatory Drugs in CF – What's New? Are They Still Needed?

James Chmiel, MD, MPH
Indiana University School of Medicine,
Indianapolis, IN

Over the past decade, tremendous progress has been made in the development of therapies directed towards the basic defect in cystic fibrosis (CF). However, CFTR modulators and similar therapies are unlikely to reverse permanently damaged airways where infection, inflammation, and mucus obstruction are already present. In addition, highly effective modulator therapies may not be available to or tolerated by all people with CF. Based upon current data available for CFTR-enhancing therapies, it is reasonable to expect that many, and perhaps nearly all, people with CF will continue to require antibiotics and anti-inflammatories for the foreseeable future.



James Chmiel, MD, MPH

Development of anti-inflammatory drugs for CF started in the 1980s with clinical trials of the oral steroid prednisone. Since the mid-1990s, dozens of anti-inflammatory drugs have been considered for CF. Unfortunately, all have fallen out somewhere along the drug development process. Newer therapies are directed towards activating the body's own ability to turn off inflammation, rather than turning off inflammation directly. Several therapies are currently in clinical trials, and thus far, the results are encouraging. This talk provides an update on the current status of anti-inflammatory drugs in the CF pipeline. <https://tinyurl.com/yxg3qaxh>

The Now and Future of Psychiatry in Cystic Fibrosis

Yelizaveta Sher, MD, FACP
Stanford University Medical Center,
Palo Alto, CA

As the effects of mental health comorbidities are increasingly recognized in people

living with cystic fibrosis (CF), mental health professionals, including psychiatrists, collaborate with and are incorporated into the CF teams. The Stanford Adult CF team is one such model where a consultation-liaison (CL) trained psychiatrist is a part of the CF team.

While mental health screenings (e.g. PHQ-9 and GAD-7) are useful starting points, an evaluation by a mental health professional is often required for an accurate diagnosis and precise treatment. Psychiatrists diagnose mental health conditions, such as anxiety, depression, bipolar affective disorder (BAD), and attention deficit hyperactive disorder (ADHD). Anxiety and depression are 2-3 times higher in people with CF; ADHD is found at higher rates (15% in one small study), and research on other mental health comorbidities is much needed. Untreated mental health

comorbidities have a negative effect on overall quality of life and medical health. Psychiatrists are able to advise CF teams on drug-drug interactions, such as combining psychotropic medications with CFTR modulators or antibiotics, and provide education to the CF teams about the mental health and emotional aspects of living with CF. They are skilled in supporting patients throughout a variety of life stages. As people with CF live longer lives and new treatments improve life quality and expectancy, psychiatrists can become important allies in mental illness and wellness for people living with CF and their healthcare teams. <https://tinyurl.com/y25x92qs>



Yelizaveta Sher, MD, FACP

ACT with CF — Overview and Applications

C. Virginia O'Hayer, PhD
Thomas Jefferson University Hospital,
Philadelphia, PA

Anxiety and depression are common among individuals with CF, and associated with decreased lung function, health-related quality of life, and increased healthcare costs. Research is lacking regarding effective and feasible interventions for these concerns among CF populations. Our 3-year

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pilot study found that Acceptance and Commitment Therapy (ACT with CF), adapted for CF patients and delivered via telehealth is a feasible treatment, reducing anxiety, depression, and cognitive fusion among CF patients, with a trend toward improved lung function. We are currently conducting a 3-year, multi-site, randomized controlled trial of ACT with CF vs supportive psychotherapy.



C. Virginia O'Hayer, PhD

Adults with CF and elevated anxiety and/or depressive symptoms are being recruited from 4 sites and randomly assigned to 6 weekly manualized sessions of either ACT with CF or Supportive Psychotherapy, delivered via HIPPA-compliant Zoom. Participants complete measures of depression, anxiety, cognitive fusion, and acceptance before treatment, after 3 and 6 weeks of treatment, and 3 months post-treatment. Lung function will be extracted from patients' EMR from 3 months prior and 3 months post-study engagement.

If ACT with CF is more effective than supportive psychotherapy in addressing the mental health and medication adherence needs among people living with CF, then we hope to continue to provide access through our ACT with CF manual. <https://tinyurl.com/y26723d7>

The Body was Made To Move

Nicole Irizarry, PT, DPT, CCS
Endurance PT, San Diego, CA

The world of cystic fibrosis (CF) is rapidly changing due to genetic modulating medication. This gives us the perfect opportunity to talk about how exercise can be used to maximize health and lon-



Nicole Irizarry, PT, DPT, CCS

gevity. A large portion of our CF community are now struggling with weight gain while others are experiencing an increase in hypoglycemia. Exercise and wellness can play a key role in these areas.

For those who are still waiting on their specific modulation medication, there is the paradox of when symptoms associated with CF may be a roadblock to being active. This presentation addresses how to start and progress an exercise program whether one's symptoms are mild or severe. Working with a physical therapist might be the missing component of reducing a person with CF's pain, improving breathing control, stamina, strength, and confidence. <https://tinyurl.com/y2j2yy78>

The Application of Phage Therapy to Cystic Fibrosis:

A Fresh Look at an Old Idea

Douglas Conrad, MD
University of California San Diego,
San Diego, CA

As CF patients age and life-long exposure to broad spectrum antibiotics increases, susceptibility to standard antibiotic therapy decreases. Bacteriophage or phage therapy offers an approach to manage these chronic infections. Phage are viruses found throughout the environment including in humans and pose little threat to their human hosts. Phage can have either a lytic or a lysogenic lifecycle. As part of their lytic lifecycle, they infect specific bacteria taxa and quickly replicate themselves, killing their bacterial host in the process.

Phage have several characteristics that make them attractive as potential therapeutic agents: a) phage are safe and can be delivered to patients with hepatic or renal failure, b) they target specific taxa such as *Pseudomonas* or *Staphylococcus*, c) they can be delivered topically to the lung via nebulization or systemically via intravenous access, d) phage can be engineered to target virulent or highly resistant members of the airway microbial community. Challenges to the widespread use of phage therapy include the standardization of the



Douglas Conrad, MD

preparation, access to well-characterized lytic phage libraries, regulatory hurdles with local institutional review boards and FDA requirements. Current access to phage therapy for CF patients is possible through unfunded, single-patient Investigational New Drug access and through a small number of clinical trials targeting *Pseudomonas aeruginosa*.

<https://tinyurl.com/y3sfrl82>

Our Patients Are Now Adults: Do We Have To Worry About Cancer?

Denis Hadjiliadis, MD, MHS
Perelman School of Medicine,
University of Pennsylvania, Philadelphia, PA

Survival for patients with cystic fibrosis (CF) has continued to increase over the last few decades and is estimated to be around 45 years in 2018. As the population is aging, a set of new issues have arisen for the CF population; in addition, higher numbers of patients with CF are immunosuppressed as a result of lung transplantation.

Cancer is a problem that has been identified, especially in patients after transplant. Gastrointestinal cancers are common either because of direct CFTR effects, issues related to pancreatic insufficiency or a combination of the above. Colorectal cancer is the most common location of malignancy and it has available screening procedures. In the transplant population, post-transplant lymphoma (PTLD) is common, in particular for patients who receive organs from donors who are Epstein-Barr virus (EBV) positive, while being EBV negative. Another cancer which is more common after transplant is skin cancer. Careful monitoring of patients at increased risk of skin cancer is recommended with mitigation of risk by using sun protection, avoiding or minimizing exposure to medications

which increase skin cancer risk, and frequent skin exams. As the CF population continues to age, cancer is likely to increase in incidence. However, the approval of highly effective CFTR modifiers could potentially decrease risk, if it is related to the presence of CF. <https://tinyurl.com/y25pljfh>



Denis Hadjiliadis, MD, MHS

Salt in My Soul

Diane Shader Smith, Los Angeles, CA

Salt in My Soul: An Unfinished Life collects diary entries of Mallory Smith — a remarkable young woman determined to live a meaningful and happy life despite her struggle with



Diane Shader Smith

cystic fibrosis and *cepacia* – from age fifteen to her death at the age of twenty-five. *Salt in My Soul* addresses issues particularly relevant to those living with or loving someone with cystic fibrosis: the power caregivers have over a patient's day-to-day quality of life; disclosure – when to tell people about your illness; fear, depression and anxiety that accompany chronic illness; body image; end of life choices; and phage therapy – a promising treatment for resistant bacteria.

At its core, this celebrated and moving memoir is a snapshot of a coming of age, an intimate and inspiring portrait of a young woman living her life, struggling with who she is, what she hopes to accomplish, and what she fears. Mallory writes about topics that so many people experience - wanting to fit in, wondering if anyone will love her, feeling insecure about her looks, grappling with personal independence, wondering what the future will look like. In doing so, she teaches us about resilience, discipline, inspiration, perspective, and insight. Mallory's mother, Diane Shader Smith, shares this powerful story using photographs, and personal anecdotes.

<https://tinyurl.com/y6bpqfmb>

Gene Corrected Autologous Stem Cells to Treat Cystic Fibrosis

Sriram Vaidyanathan, PhD
Stanford University, Palo Alto, CA

The development of CRISPR/Cas9 has enabled the precise modification of the human genome. The combination of Cas9 with adeno-associated viruses (AAV) has been most effective in the gene modification of primary stem cells. We pursue an ex-vivo gene correction strategy in which mutated stem cells are genome edited outside of the body and then transplanted back into the patient to provide durable restoration of CFTR function.

We first reported the high-efficiency correction of the F508del mutation in airway basal stem cells isolated from the upper airways (nose and sinuses) and bronchi using CRISPR/Cas9 and AAV. We obtained correction rates of >30% in airway basal stem cells and we observed 30-60% restoration of CFTR function relative to non-CF controls in airway epithelial sheets differentiated from corrected basal stem cells. We are further extending these methods to insert the full coding sequence of CFTR and thus correct almost 100% of CF mutations, including the variants that cannot be treated by modulators. Since the CFTR coding sequence with homology arms is too large to be packaged in one AAV vector, we sequentially insert two halves of the CFTR cDNA along with a marker that enables the enrichment of corrected cells (truncated CD19, tCD19). In experiments with airway basal stem cells from 11 different CF donors, we observed ~70% corrected tCD19+ stem cells after editing and enrichment. On differentiation of corrected airway basal stem cells, we observed >70% restoration of CFTR function relative to non-CF controls. These experiments are an important first step for development of an autologous genome edited airway stem cell therapy for CF.

<https://tinyurl.com/y4y3b4d4>

Cystic Fibrosis and Sleep

Caroline Okorie, MD, MPH
Stanford University School of Medicine,
Palo Alto, CA

Poor sleep is associated with lower mood, decreased immune function, heart disease, poorly controlled diabetes and overall lower quality of life. From a respiratory standpoint, sleep is a vulnerable time associated with a decreased ability to exchange respiratory gasses (i.e. ventilation), decreased lung volume for each breath, and increased upper airway resistance. Therefore, it is especially important for those with underlying pulmonary conditions, like CF, to be mindful of how their sleep affects their overall health. The various causes of poor sleep in the general population include

stress, schedule demands, electronics, and poor sleep habits. Patients with CF experience additional factors, including chronic cough, pain, heartburn, frequent stooling and medication side effects. More than half of all CF patients have sleep complaints and the frequency increases with severity of lung disease.

Sleep logs, actigraphy and sleep studies are all tools available to measure sleep quality. Conditions such as obstructive sleep apnea, restless legs syndrome, and insomnia can further decrease the sleep quality (and overall health) of patients with CF. A sleep study can help detect early signs of decreased ventilation and hypoxemia in patients with CF lung disease. Early detection can result in early treatment with non-invasive ventilation or supplemental oxygen. Insomnia can also affect patients with CF. For chronic insomnia, cognitive behavioral therapy is now considered the gold standard for treatment. Since we know how important good quality sleep is to overall health, it is important for sleep to be a part of the discussion in any healthcare maintenance visit.

<https://tinyurl.com/y6725yul>

Learning, Laughing, and Loving with CF

Julie Desch, MD, San Rafael, CA

Julie travels at near light speed through her life of almost sixty years of living with cystic fibrosis, describing the highs and lows as remembered through a very

hazy rearview mirror. Beginning with her childhood persona named “Bub” who lived in a story remembered as sad and fearful, she describes how this was actually a time of great resilience training. Moving into the years of early adulting and becoming a



Caroline Okorie, MD, MPH



Sriram Vaidyanathan, PhD



Julie Desch, MD

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From Yoga to Physical Therapy: Virtual CF Wellness Programs Improve Physical and Mental Health

CFRI's wellness programs bring to life the positive impact of movement and exercise upon one's physical and mental health. These online programs are free, fun and interactive, and are open to those with CF, as well as their parents, spouses, partners and siblings nationwide. In addition to a Physical Therapy class with Nicole Irizarry, DPT, and Karen Von Berg, DPT, we offered strength building classes with Taylor Lewis, MA, and Yoga classes taught by Martha Modawell, Certified Yoga Instructor and mother of two daughters with CF, and Colleen Lewis, Certified Yoga Instructor and adult with CF. Our instructors and class participants log in from throughout the United States. We will offer a final yoga class in the fall of 2020 led by Colleen Lewis. By exercising together, our community builds emotional and physical resilience, while forging new connections and friendships.

For the current schedule, go to the events page at cfri.com.

CFRI's CF Wellness Initiative is supported to date through a grant from Vertex Pharmaceuticals and InterWest Insurance Services, LLC.



CFRI Is Your Partner in Living

Some Easy Ways to Deepen Your Relationship

- **HOLD YOUR OWN VIRTUAL EVENT:** Cocktails for a cure, yoga, Pictionary challenge – no idea is too big or too small. Create an event, 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!
- **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 Stacie Reveles (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 Stacie Reveles, CFRI's Advocacy and Programs Associate: 650.665.7586 or sreveles@cfri.org.



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.

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April 1, 2020 — September 15, 2020



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April 1, 2020 — September 15, 2020



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Pet Memorials:

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A Multi-'Omic Approach

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or disease status, the role of the respiratory microbiota in diseases of the airways is still poorly understood. Recent studies of the lung microbiota of cystic fibrosis (CF) patients have shown that a diverse microbiota colonizes the CF lung during childhood and that the composition of the lung microbiome changes, and diversity decreases, as the disease progresses. The upper airways are also affected by the defect in the CFTR gene (causing CF disease), and because of this, CF patients commonly present with chronic rhinosinusitis (CRS).

Previous studies of CF patients with CRS have shown that the upper airways are colonized by identical strains of a major CF-pathogen, *Pseudomonas aeruginosa*. A recent study of the upper and lower airway microbiome in CF-CRS patients using marker gene sequencing demonstrated a high degree of similarity in the airway microbiome of CF-CRS patients, but strain-level resolution could not be achieved. These findings suggest the possibility of the sinus as the portal of entry for bacterial pathogens and other microbes into the lungs. However, it is still unclear how sinus microbiota affects the lung microbiota composition and lung inflammation. In our studies, we use a multi-'omics approach to robustly characterize the sinus and lung microbiota and determine the functional attributes of microbes that correlate with inflammation in the upper and lower airways.

Our research team has made good progress on the metagenomics analysis and we have demonstrated a unique bacterial and fungal signature in the upper and lower respiratory tracts of study participants with diagnosed CF and CRS. These studies will help to determine which microbes or their functions contribute to CF disease progression and will help direct future studies to define candidate microbes for which to develop targeted microbiome-based therapeutics against. Our study will help uncover the role of the upper and lower airway microbiome in CF patients and lead to better clinical management of this devastating disease.

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doctor, times got both better and worse, depending on the day. Middle age followed, because despite her doctor's somber observation, "You should be dead," she continued to wake up day after day assuming the role of Mommy, partner and CF volunteer. This brings us to present day, where the magic of Trikafta has completely changed the game, the role of Drill Sergeant has been dropped, and life is simply lived and enjoyed. Julie hopes to both entertain and motivate with stories from the trenches of identity building and loss, family disruption and generation, mental and physical health challenges and the lessons they taught, and how the tools of exercise, meditation, humor and love has sustained a life that could not be more full.

<https://tinyurl.com/y2xs9omj>

Advocacy During the Pandemic



COVID-19 has had a devastating impact upon our cystic fibrosis community on many levels. While all 2020 in-person events were cancelled, CFRI has continued to raise awareness and advocate on behalf of the CF and rare disease community through virtual meetings with elected representatives, action alerts on pressing legislative issues, and informational updates on policies and legislation that impact our community.

CFRI has advocated independently and in partnership with other health-related organizations at the state and federal level to address discriminatory medical rationing - urging the removal of language from state emergency plans that excludes or deprioritizes those with advanced lung disease from receiving ventilators during a shortage. We have urged our community members to express their opinions related to access to telemedicine, and expansion of paid medical leave.

CFRI endorsed California Proposition 14 – Californians for Stem Cell Research, Treatments and Cures – so as to continue the California Institute for Regenerative Medicine, which has funded extraordinarily promising research for the CF community.

CFRI is raising awareness to halt the rapid expansion of Co-Pay Accumulator Programs, which no longer allow patients to apply their co-pay cards from drug manufacturers toward their annual deductible. Once patients exhaust their co-pay coupons, they discover that they still have hundreds or thousands of dollars due in out-of-pocket payments before their insurance coverage applies. Four states have outlawed these programs and federal legislation has been introduced to allow co-pay accumulator programs only when a generic drug equivalent is available. We will keep you informed.

CFRI remains firmly opposed to the use of the Quality Adjusted Life Year (QALY) to measure drug value and price. The use of QALY originated as a means to ration care, and it is inherently discriminatory toward people with chronic disease and disabilities. The use of QALY to assess medication pricing for the CF and rare community is unethical, subjective, and likely a violation of the Americans with Disabilities Act.

CFRI will continue to keep our community informed, supported and engaged as we cope with the challenges of COVID-19 and life with a challenging rare disease.

2020 CFRI Award Recipients

CFRI proudly presents these annual awards in recognition of outstanding contributions to the CF community. We thank these remarkable people for their time and their commitment to those living with cystic fibrosis.



Oscar Flamenco, CPA

Dave Stuckert Memorial Volunteer of the Year Award — Oscar Flamenco, CPA

As Treasurer on CFRI's Board of Directors, Oscar Flamenco plays a significant role in CFRI's strategic success. Oscar is married to a woman with CF who received a double lung transplant. Despite juggling two demanding jobs, Oscar unfailingly shares his time to support CFRI, helping CFRI to maintain a 4-star rating with Charity Navigator, and ensuring CFRI's financial stability. While Oscar's work may not be overtly visible, our community benefits from the many hours he shares.

Professional of the Year Award — Colleen Dunn, MS, RRT, CCRC

Colleen Dunn is a CF Clinical Research Coordinator at Stanford, where she has earned national and international prominence with the CF clinical trials network. Colleen has trained research coordinators nationwide and serves on Stanford's Pediatric and Adult CF Advisory committees, as well as on CFRI's Board of Directors and Research Advisory Committee. Due to her multi-faceted contributions to CF, Colleen has changed people's lives and helped to advance CF research.



Colleen Dunn, MS, RRT, CCRC

CFRI Partners in Living Award in Memory of Anabel Stenzel — Todd Giebenhain

Todd Giebenhain is an actor, who has had roles in dozens of well-known shows. An adult with cystic fibrosis, Todd serves with dedication on CFRI's CF Summer Retreat Committee, where is a vocal advocate for inclusivity for CF adults, seeking to decrease isolation and create community. Todd embodies the spirit of this award – due to his initiative, determination, adherence to his medical regimen, positive coping, and engaged community service.



Todd Giebenhain

Paul M. Quinton Cystic Fibrosis Research Legacy Award — Beate Illek, PhD

Beate Illek, PhD, is Assistant Scientist and Director of the Cystic Fibrosis Research Laboratory at Children's Hospital Oakland Research Institute. Her laboratory studies salt and water transport across epithelial tissues. Dr. Illek and her team identified a number of small molecules that target the CFTR chloride ion channel including genistein and other flavonoids, which paved the way for CFTR-based drug development. Dr. Illek is the volunteer director of CFRI's Elizabeth Nash Memorial Fellowship Program and a strong advocate for inspiring researchers to join the field of CF.



Beate Illek, PhD

Thank you to Our Community Sponsors and Donors for CFRI's A Breath of Fresh Air Gala!

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For complete list, see Gala info on Back Cover

**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
November 17 / December 15
January 19 / February 16
March 16 / April 20

Go to www.cfri.org for information
Participate by Zoom or phone

Sponsored by Vertex Pharmaceuticals,
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and Private Individuals

**Online Support Group
for Adults with CF**

Third Monday of Every Month
November 16 / December 21
January 18 / February 15
March 15 / April 19

Go to www.cfri.org for information
Participate by Zoom or phone

Embrace Mothers' Retreat

May 1 – May 2, 2021

**CFRI 34th National CF Education
Conference ~ A Virtual Event**

July 30 – August 1, 2021

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

If you have not signed up to receive our weekly eNewsletter, go to www.cfri.org to sign up! You will always be up to date on the latest news.



36th Annual Golf Tournament Benefitting CFRI – Socially Distant Yet Successful!

On August 10, the world-famous Pasatiempo Golf Club in Santa Cruz, CA – a “Top 100” course – hosted a group of dedicated golfers who enjoyed friendly competition while supporting the search for a cystic fibrosis (CF) cure. This year’s event was modified due to COVID-19 and followed strict guidelines for social distancing and infection control. Participants had an incredible day, raising over \$65,000 for CFRI! Of this total, \$15,000 will be matched by CFRI’s Jessica Fredrick Memorial CF Research Challenge Circle and designated for CFRI’s research grant awards.

The event is deeply personal for the event co-chairs, Scott Hoyt and Mike Roanhaus – both have daughters who have cystic fibrosis. CFRI is extremely grateful to Scott, Mike, and the dedicated members of the event committee, Francine Bion, Tina Capwell, and Ralph Swanson, and the many participants whose support advances cutting-edge research and much needed support programs for those living with CF. We also thank the long-time major sponsors of the event - Star One Credit Union, the Kirkorian Family Foundation, as well as the Mike and Dea Roanhaus family. Dates for 2021 will be announced soon!

From a COVID-19 Playlist to CF Mental Health: CF Community Voices Has Something for Everyone



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, including CF and stem cell research, COVID-19 and CF, all conference presentations, ototoxicity, and more. New episodes are released monthly, and can be watched on CFRI’s YouTube channel or downloaded from cfri.podbean.com. We look forward to sharing our community’s diverse voices.

Generously sponsored by Vertex Pharmaceuticals, Chiesi USA, Gilead Sciences, Genentech, and Mylan.

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

The COVID-19 pandemic has worsened symptoms of depression and anxiety for many in the cystic fibrosis (CF) community. With its unpredictability, daily treatment burden, and diverse symptoms, CF is a challenging disease for those diagnosed, as well as for those who love them. 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 CF social workers from Stanford. The groups are held via Zoom for the nationwide community.
- **CF Adult Support Groups:** Adults with CF are invited to this group, held the third Monday of every month and facilitated by CF social workers. The groups are held via Zoom.
- **“Living Mindfully with CF” Online Classes:** Mindfulness Based Stress Reduction practices are focused on life with CF, helping to reduce anxiety and depression. Offered to individuals with CF and their family members, the class is taught by Julie Desch, MD, who herself lives with CF.
- **CF Bereavement Group:** For those who have lost a loved one to CF, this online group includes sharing and discussion, journaling/writing, goal setting, grief education, and self-care strategies, and is led by Isabel Stenzel Byrnes, LCSW, MPH, bereavement social worker 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, individual donors, 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

Members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle give generously to inspire others to join the search for new CF therapies and a cure. This year to date, Circle members have contributed over \$90,000 so as to match – dollar for dollar – donations from individuals committed to CF research. Together, these donations are used for our CF research awards.

Our Circle is named in memory of Jessica Fredrick, who lost her battle with CF at the

age of 21. There is still no cure for CF. We need your help to improve and save the lives of our loved ones. Please join this inspiring group! Become a member of the Jessica Fredrick Memorial CF Research Challenge Circle by making a minimum gift of \$2,500. You will inspire others to make the dream of a CF cure a reality.

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.





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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

To be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support.

CFRI Vision

To find a cure for cystic fibrosis while enhancing quality of life for the CF community.

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

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

Visit our website at:

www.cfri.org

for more information about us and about cystic fibrosis.

Call toll free: 855.cfri.now

CFRI's A Breath of Fresh Air Virtual Gala An Astounding Success!

CFRI's annual gala, held virtually on October 17, 2020, brought together a community united in the search for a cure for cystic fibrosis. A lively pre-gala event was attended by community members, nationwide, led by Emily Schaller, CF warrior and leader of the Rock CF Foundation. The official program launched with a welcome from Olympic champion Scott Hamilton, while our emcee, Chris Chmura of NBC Bay Area, guided us through a moving program in which the experiences of our diverse CF community members were shared. Mary Helmers, RN, of Stanford's CF Center, was honored as our 2020 CF Champion. Ultimately, over \$127,000 was raised, and \$40,000 of this total will be matched dollar-for-dollar by members of CFRI's Jessica



Fredrick Memorial CF Research Challenge Circle and designated for research. Warm thanks to all who participated! Mark your calendars for our 2021 *Breath of Fresh Air* event on October 16, 2021.

We are grateful for our generous sponsors, in-kind donors, attendees and hardworking Gala Committee members. Everyone played a role in our virtual gala's amazing success – it was truly *A Breath of Fresh Air!*

For complete list of Gala sponsors and donors see page 9

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