CFRI community
46 Years of Research, Education, Advocacy, & Support
Fall 2021

CFRI's 34th National Cystic Fibrosis Education Conference: Presentation Summaries

By Siri Vaeth, MSW

At CFRI's 34th National CF Education Conference, Partners in Progress, held virtually July 30 to August 1, 2021, 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 summaries below are derived from presenter abstracts. On page 6 readers will find a link to CFRI's 2021 Conference Presentation Playlist.

Breath from Salt

Bijal P. Trivedi, MSc, MA
National Geographic, Washington, DC

In 2011, Bijal Trivedi knew little about cystic fibrosis, and had never met anyone who suffered from it. It was only once she was assigned to write a magazine feature on Kalydeco, in 2012, that she unwittingly began a journey to tell the complete story of this disease: from its characterization to the discovery of the defect in the cell and gene to the development of the amazing medicines that are available today. And while the science was always path-breaking, it was the individuals in this story who captivated her, won her heart, and made her realize that she needed to write a book about this epic saga. In this presentation, Bijal tells about her decade-long journey writing this book, some of the people who inspired her, and shares selected excerpts of *Breath from Salt*.

Getting to the After Times – CF and COVID in Year 2

Richard Moss, MD
Stanford University, Palo Alto, CA

In this update, Dr. Moss addresses many key questions about the pandemic that are relevant to those with cystic fibrosis: How have people with CF fared? Is having CF a vulnerability for COVID? How effective are the vaccines in protecting from serious illness or death? How safe are the vaccines, and are there important differences between them? How common are breakthrough infections to those vaccinated? What are variants

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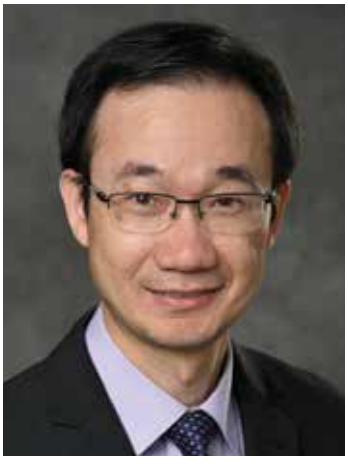

Role of Disrupted Airway-Surface Liquid pH Regulation in Small Airways in Cystic Fibrosis

By Xiaopeng Li, PhD / Michigan State University

Cystic fibrosis (CF) is caused by mutations in the gene that encodes the CF transmembrane conductance regulator (CFTR) anion channel. In CF patients, the loss of CFTR impairs airway host defense function and leads to chronic airway infection and inflammation, which is the main causes of morbidity and mortality. It is generally believed that small airways are involved early in the pathogenesis of CF lung diseases. Therefore, understanding the mechanisms underlying small airway host defense defects is of great importance to advancement of CF therapy.

It was assumed that the mechanisms underlying host defense defects in the small CF airways are the same as in the large airways. However, our studies have shown that there are distinct differences

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CFRI Community
Fall 2021

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Letter from the Executive Director

Dear Friends,

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 and terrifying weather events. It remains a very challenging time.

This year, CFRI celebrates 46 years of research funding and service to our community. With your support, we have grown tremendously since 1975, funding innovative CF research while incorporating education, psychosocial support and advocacy programs. CF remains a harsh and capricious disease, and many members of our community – particularly people of color – are still waiting for transformative therapies. CFRI is proud to fund innovative research to move us closer to a cure.

In 1977 we provided our first grant to Dr. Paul Quinton, a legend in the field of CF research whose work transformed our understanding of the disease. Thanks to the efforts of our Research Advisory Committee, which reviews and vets proposals, we are supporting the work of many inspirational scientists. The funding provided by CFRI is often the spark that lights a larger funding flame. In this issue you will see what incredible work is taking place in labs across the country.

As we approach the end of 2021, let us reaffirm our collective commitment to the work that remains to be done. Thank you for your consistent generosity and support.

Warm regards,

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

News from the Board

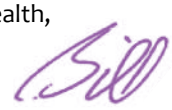
Dear CFRI Community,

I hope that you are safe and well as the challenges presented by COVID-19 continue. There are no "silver linings" when speaking of a pandemic, but as President of CFRI's Board of Directors, it has been extremely uplifting to witness our community's resilience and spirit throughout these past 20 months. Together we have helped to advance CF research while continuing our commitment to programs that enrich the lives of all impacted by CF. The Board of Directors and staff have been very creative in seeking new sources of revenue so as to maintain our quality services. As you will see in the enclosed Annual Report, our recent audit once again confirms that we are a financially stable organization with excellent scale management.

As you read this, members of CFRI's Research Advisory Committee are in the process of evaluating Letters of Intent for our next round of Elizabeth Nash Memorial Fellowship and New Horizons Program research awards. This newsletter contains several abstracts describing the innovative work currently pursued by CFRI-funded researchers. As a member of CFRI's community, you play a key role in bringing this innovative research to life.

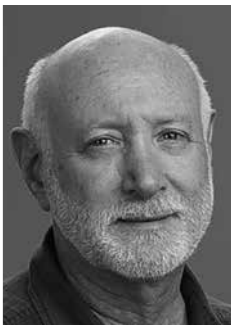
Thank you for your ongoing support. With your help we continue to move closer to a cure.

Peace and good health,



Bill Hult | *President, CFRI Board of Directors*

Bijal P. Trivedi, MSc, MA



Richard Moss, MD

of concern, and how well do the vaccines protect against them? Can vaccinated people spread the virus to unvaccinated others? Do we need to vaccinate children? What about transplant recipients and the immunocompromised? Will CF care be changed by our adaptations – for example are telehealth and home monitoring here to stay? Are there medicines that effectively treat COVID? What about booster shots? How common and serious are chronic "long haul" COVID symptoms? In his presentation, Dr. Moss reviews current evidence and recommendations to address all of these questions and beyond.

Advances in Phage Therapy as a Treatment for Cystic Fibrosis

Benjamin Chan, PhD; Jonathan Koff, MD
Yale University, New Haven, CT

Benjamin Chan, PhD

There has been renewed interest in bacteriophage (phage) therapy, which has been used to treat infections since their discovery in early 1900s. Phages are viruses that specifically infect and kill bacteria; they are ubiquitous and found wherever bacteria are present. In addition, there is an opportunity to find naturally occurring lytic phages that kill CF pathogens (e.g., *Pseudomonas aeruginosa* and *Staphylococcus aureus*), or potentially to genetically modify these phages to improve their function. In their presentation, Dr. Chan and Dr. Koff provide an overview of phage therapy while sharing study results with a cohort of 9 adults with CF in which phage nebulized daily for 5 to 10 days decreased *Pseudomonas sputum* bacteria load significantly. In addition, there was evidence for 1) increased antibiotic sensitivity in individuals that received efflux pump-targeting phage, 2) decreased pilus function, which resulted in less virulence, and 3) an improvement in lung function after phage therapy. These results prompted the development of a clinical trial [CYstic fibrosis bacterioPHage study at Yale (CYPHY)], funded by the CF Foundation and Yale University, to investigate the safety and efficacy of this nebulized personalized phage therapy approach, and the establishment of a Center for Bacteriophage Research and Therapy at Yale.

Jonathan Koff, MD

mRNA Therapy as a Treatment for CF

Deepika Polineni, MD, MPH
University of Kansas Medical Center, Kansas City, KS

Deepika Polineni, MD, MPH

It is estimated that for at least 9% of people with CF, recently approved CFTR modulator treatments are not applicable based on genotype or adverse effects to therapy, hence alternative strategies to improve CFTR function are needed. Messenger ribonucleic acid (mRNA) therapy presents a cutting-edge treatment option under investigation for people with CF, independent of CFTR mutation. mRNA is a type of ribonucleic acid that is present in human cells and represents one step in the process of DNA genetic code becoming translated to protein. Using mRNA as therapy in CF involves the careful delivery of mRNA, coding for CFTR, into the cell to use the cells' machinery to create normal CFTR protein. Notably, mRNA therapy is not the same as either gene editing or gene therapy. However, mRNA has the potential to address all people with CF irrespective of their CFTR genotype. This presentation provides a review of mRNA as a novel therapeutic option for people with CF of all mutation types, and results of the most recent clinical trials evaluating mRNA therapy in CF are discussed.

Pulmonary Exacerbations in the Era of Highly Effective CFTR Modulators

D.B. Sanders, MD, MS
Indiana University School of Medicine, Indianapolis, IN

D. B. Sanders, MD, MS

The introduction of highly effective CFTR modulators has greatly reduced the frequency of pulmonary exacerbations. However, they have not been eliminated entirely, and the long-term impact of highly effective CFTR modulators on pulmonary exacerbations will not be known for years. Furthermore, many people with CF are not eligible, or cannot tolerate, highly effective CFTR modulators. Research efforts are ongoing to optimize the treatment of pulmonary exacerbations. The recently completed STOP-2 trial provided valuable information on the benefits of varying durations of intravenous antibiotics. STOP PEDS is exploring whether children with CF with mild pulmonary exacerbations can avoid antibiotics entirely, especially if they are receiving highly effective CFTR modulators. The impact of highly effective CFTR modulators may mean that "a cold is just a cold," and not something that can lead to weeks of symptoms, missed school or work, or prolonged hospital stays. This talk provides an overview of these potential benefits and how the CF community is preparing to treat respiratory symptoms in this new era.

Advancing the GI Frontier for People with CF

Steven Freedman, MD, PhD
Beth Israel Deaconess Medical Center, Boston, MA

Steven Freedman, MD, PhD

As we are making major strides in addressing and improving lung disease in patients living with CF, the GI manifestations in these patients are now being recognized as a

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major cause of symptoms. In fact, multiple studies show that approximately 80% of patients living with CF have GI issues - from acid reflux to motility disorders such as gastroparesis, as well as constipation and DIOS. These issues are frequently moderate to severe and impact quality of life. Exocrine pancreatic insufficiency is an early manifestation in over 90% of patients with CF and was thought to be irreversible. However, studies indicate that exocrine pancreatic function can potentially be rescued with highly effective CFTR modulators, at least up to age 5. Similarly, recurrent pancreatitis in pancreatic sufficient patients with CF may be prevented with Ivacaftor. There is an increased risk of colon cancer associated with CF. Whether Trikafta can reverse the CF manifestations across all organ systems in patients age 12 and older, is being examined through the 2 year long PROMISE trial.

Three Perspectives, One Purpose: Why Medicine Needs Memoir

Diane Shader Smith; Maryanne O'Hara; David Weill, MD
Los Angeles, CA; Ashland, MA; New Orleans, LA


Diane Shader Smith

Patient stories offer valuable insights that go way beyond statistics and outcomes: they have the power to inspire, humanize, compel action, and challenge assumptions. Patients want to tell their stories, but how do you choose the right stories, and how do you make sure that the process of telling and hearing them is beneficial to both provider and patient? Diane Shader Smith, Maryanne O'Hara and David Weill, will speak for the patient, the parent, and the provider respectively, and address these questions in the context of cystic fibrosis. Using David's memoir, Exhale: Hope Healing and a Life in Transplant, Maryanne's memoir, Little Matches: A Memoir of Grief and Light, and Mallory Smith's memoir, Salt in My Soul: An Unfinished Life, they will discuss the role of narrative in medicine as it relates to the parent-provider relationship, bioethical issues affecting both privileged and underserved communities, systemic failings, scientific inquiry, and the role of palliative care from the time of diagnosis until end of life.


Maryanne O'Hara


David Weill, MD

Strength-Building with CF

Taylor Lewis, MA, CSCS
Pulmonary Performance Institute, San Rafael, CA


Taylor Lewis, MA, CSCS

A common supplemental approach to improving the impacts of cystic fibrosis is the integration of physical activity. Research has shown that strength and conditioning-based fitness programs are very beneficial for individuals with CF. Nonetheless, there needs to be more in-depth discussions about how strength training can build a strong foundational support for physical activity and fitness sustainability. Peripheral muscle weakness is prevalent in adults with cystic fibrosis. Strength training enhances the capacity of individuals to absorb and redistribute mechanical forces that improves physical fitness. The optimization of mechanical force production allows individuals to increase workloads in aerobic and anaerobic-based settings resulting in improved performance outcomes. Research has shown that strength training, as a complementary approach to endurance training, can improve health outcomes and quality of life. Paralleling strength training with conditioning-based training programs is key to improving and sustaining exercise capacity and functional fitness performance levels in CF.

CFRD Management Through Technology

Amir Moheet, MBBS
University of Minnesota, Minneapolis, MN


Amir Moheet, MBBS

Cystic fibrosis-related diabetes (CFRD) is the most common extra-pulmonary complication of cystic CF. Around 40% of individuals with CF above age 20, and 50% above age 30 have CFRD, which is associated with increased morbidity and mortality. The current guidelines recommend insulin as the only pharmacological therapy for management of CFRD. Patients with CFRD are asked to monitor glucose levels which requires frequent finger stick glucose monitoring. Multiple daily insulin injections and frequent finger stick glucose monitoring ads to the already high treatment burden in these patients. Recent advancements in diabetes technologies – including ambulatory insulin pumps, continuous glucose monitoring (CGM) and hybrid closed-loop insulin delivery systems – have transformed the landscape of diabetes management. These technologies are being increasingly utilized in the management of CFRD. In this presentation, current insulin pump, CGM and hybrid closed loop insulin options are discussed, and the potential benefits of these technologies in the management of CFRD reviewed.

Until It's Done for Everyone: Diversity, Inclusion and Equity in CF Care and Research

Jennifer Taylor-Cousar, MD, MSCS
National Jewish Health, Denver, CO


Jennifer Taylor-Cousar, MD, MSCS

The characterization of CF as a "White" disease has contributed to late diagnosis and treatment in Black, indigenous and people of color (BIPOC) with CF. This is associated with worse health outcomes including lower lung function and increased risk of death, and contributes to medical trauma

and compounds distrust of the medical system. Both medical system distrust and investigative team implicit bias likely explain the disproportionately low enrollment of BIPOC with CF in clinical trials. While more than 90% of White people with CF (pwCF) are eligible for a CFTR modulator, only 76% of Hispanic and 70% of Black pwCF are eligible for these life-changing therapies. In vitro evaluation of use of modulators for CFTR mutations more commonly found in BIPOC with CF and not previously included in testing efforts is underway. Recent acknowledgment of health care disparities in CF by CF organizations and within the CF community is an important first step in addressing the institutional and interpersonal factors that uniquely adversely impact the quantity and quality of life for BIPOC with CF. Efforts to increase representation of diverse voices from the CF community in care and research and CF workforce diversity are important additional steps and are ongoing. Intentionality in our endeavors is crucial to ensure that CF is ultimately cured for everyone.

Hearing is Believing: Hearing Health in Persons with Cystic Fibrosis

Angela Garinis, PhD, CCC-A;
Ahmet Uluer, DO, MPH
Oregon Health & Science University, Portland, OR; Boston Children's Hospital/ Brigham & Women's Hospital, Boston, MA

Angela Garinis, PhD, CCC-A

Ahmet Uluer, DO, MPH

Persons with cystic fibrosis (CF) are routinely treated with aminoglycoside and glycopeptide antibiotics to manage life-threatening bacterial infections. Potential adverse events to the auditory system from these treatments (ototoxicity) may include hearing loss, tinnitus, vertigo and even speech in noise processing issues. Susceptibility to ototoxicity may vary across patients due to various risk factors such as genetic markers for hearing loss, noise exposure and other concomitant treatments beyond aminoglycosides and glycopeptides administered intravenously. This presentation discusses: (i) the pathogenesis of CF with focus on the micro-biome and use of ototoxic treatments (e.g., aminoglycosides) (ii) synergistic ototoxic effects (e.g., noise, inflammation, other treatments) (ii) ototoxicity monitoring and management options for CF care centers and (iv) and provides a brief discussion on novel clinical ototherapeutic trials.

Advances in Gene Therapy and Animal Models for Cystic Fibrosis

John Engelhardt, PhD
University of Iowa, Iowa City, IA

John Engelhardt, PhD

The two primary approaches for CF gene therapy include gene replacement (the addition of a functional CFTR gene or mRNA) and gene editing (the correction of CFTR mutations within the patient's DNA or mRNA). Each of these approaches require delivery vehicles (vectors) that move genetic cargo into cells of the target organ. Several vector systems are currently being pursued including lipid nanoparticles, RNA viruses (lentivirus), and DNA viruses (adeno-associated virus and human boca virus). Animal models of disease are integral to the developmental pipeline of gene and cell therapies. For two decades, CF mice had been the only animal model available in which to develop therapeutics. More recently, however, new CF animal models have been developed, including pigs, ferrets, sheep, rabbits, and rats. CF is a complex disease and the path to developing effective gene therapies will likely encounter unknown challenges and setbacks. However, many companies and academic laboratories have contributed significant resources and talent to tackling these challenges and ensure eventual success.

Reproductive Health in Men and Women with CF: What Do We Know and What Do We Need to Know?

Raksha Jain, MD, MSc
University of Texas Southwestern, Dallas, TX

CFTR protein dysfunction results in abnormal chloride and bicarbonate transport in

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Role of Disrupted Airway-Surface Liquid pH Regulation in Small Airways in Cystic Fibrosis *Continued from Cover*

between small and large airways including epithelial morphology and cell types compositions, airway-surface liquid (ASL) pH and viscosity, and bacterial killing. In a newborn pig CF model, the ability of gland-containing airways to fight infection was affected by at least two major host-defense defects: impaired mucociliary transport and a lower ASL pH. In the gland-containing airways, ASL pH is balanced by CFTR and ATP12A, which respectively control HCO_3^- transport and proton secretion.

We found that although porcine small airway tissue expressed little ATP12A, the ASL of epithelial cultures from CF distal small airways (diameter <200 µm) were nevertheless more acidic (compared to non-CF). Therefore, we hypothesized that gland-containing airways vs. small airways control acidification using distinct mechanisms. Our microarray data suggested that small airway epithelia mediate proton secretion via ATP6V0D2, an isoform of the V0d subunit of the H+-translocating plasma membrane V-type ATPase. Immunofluorescence of small airways verified the expression of the V0d2 subunit isoform at the apical surface of Muc5B+ secretory cells, but not ciliated cells. Inhibiting the V-type ATPase with bafilomycin A1 elevated the ASL pH of small airway cultures, in the presence or absence of HCO_3^-, and decreased ASL viscosity.

These data suggest that, unlike large airways, which are acidified by ATP12A activity, small airways are acidified by V-type ATPase, thus identifying V-type ATPase as a novel therapeutic target for small airways diseases. We will investigate if V-type ATPase regulates ASL pH in human small airway and if blockade of V-type ATPase activity in human small airway will have beneficious effect on host defense function.

Dr. Li's research is being conducted with the support of CFRI.

CFRI's 34th National Cystic Fibrosis Education Conference *Continued from page 5*

Raksha Jain, MD, MSc

the respiratory, gastrointestinal, and also in the reproductive tracts. As people with CF are leading longer and healthier lives, sexual and reproductive health is becoming an increasingly important topic with a number of unique and unaddressed questions. It is critical that we provide high quality evidenced-based data to men and women with CF on topics including contraception, fertility, pregnancy, lactation, and parenthood. This session highlights what is known and unknown about male and female infertility in CF, contraception and its impact on health in CF, and pregnancy, lactation, and parenthood for men and women with CF. Importantly, this topic is discussed in the context of highly effective CFTR modulators, which are now available for over 90% of people with CF.

My Life with Cystic Fibrosis, Our Unlocked Futures, and Breaking Down Barriers for the Continued Success of the CF Community

Gunnar Esiason, MBA
Boomer Esiason Foundation, New York, NY

Gunnar Esiason, MBA

Gunnar Esiason describes his journey with cystic fibrosis from childhood to a newly minted Masters in Business Administration. Gunnar was diagnosed with cystic fibrosis when he was two years old at Cincinnati Children's Hospital; soon after, his parents founded the Boomer Esiason Foundation. Initially set up to raise money for research, the organization eventually evolved into philanthropy aimed not only at advancing cystic fibrosis science, but also to provide financial assistance and resources to people with cystic fibrosis and their families at various lifetime milestones, like college scholarships, transplant cost assistance, disaster relief, and, most recently, pandemic economic hardship. Gunnar grew up in a time with cystic fibrosis when the standard of care had very little variation from patient to patient. By 2018, Gunnar stood on the precipice of end-stage illness after living through a downward cycle requiring intervention or hospitalization every other month, if not more frequently. It was then he enrolled in a pivotal clinical trial that saved his life. Gunnar's eyes have turned to the future of cystic fibrosis, where the next generation of patients will not have to grow up with the same disruptions that Gunnar and patients in his generation have had to endure.

Recordings of these presentations are available on CFRI's YouTube channel: Go to **https://www.youtube.com/c/CysticFibrosisResearchInstitute/featured** and click on the 2021 Conference Playlist.

CFRI's 34th National CF Education Conference was sponsored by Vertex Pharmaceuticals, Genentech, Gilead Sciences, Chiesi USA, AbbVie, and Ionis Pharmaceuticals.

New Diversity Awareness Films Released: More to Come By Year End

CFRI is committed to raising awareness of the diversity of our community so as to provide support and address disparities that arise due to the misperception that cystic fibrosis only impacts those of European descent. CFRI's very active Diversity and Inclusion Advisory Committee has been creating meaningful resources for underrepresented groups, while raising diversity awareness within our community and the general public.

As part of our work, we have released two films to date addressing CF in the South Asian community and CF in the African American community. Both films feature members of our community who share their personal experiences with late diagnoses despite classic manifestation of cystic fibrosis, and the need for heightened awareness of CF among communities of color, the medical profession, and our own community.

In addition to these films, CFRI is building video playlists on our YouTube channel for those who speak Spanish or Hindi, to provide information, resources and support. To view the films and playlist, go to our YouTube channel: **https://www.youtube.com/c/cysticfibrosisresearchinstitute**

Support for CFRI's Faces of CF Diversity and Inclusion Program is provided by Vertex Pharmaceuticals, Gilead Sciences, Genentech, and Chiesi USA.

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:

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Or go to **www.cfri.org** to make a donation online.

In Honor of

April 1, 2021 — September 30, 2021

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April 1, 2021 — September 30, 2021

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Pam Wood

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 on the third Tuesday of every month 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.

— **Transplant Support Group:** This group is open to post-transplant CF adults nationwide. Meetings are held on the fourth Wednesday of every month, starting October 27, and will be facilitated by Sonya Haggett, LCSW, transplant recipient with CF.

— **"Mindfulness 2.0" Online Classes:** The course is based on Unified Mindfulness and has been adapted for the CF community. It aims 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.

— **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. We are hoping to add additional support groups in 2022. 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, individual donors, and contributions through CFRI's CF Quality of Life Program, a Living Legacy of Peter and Kathy Judge.

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 family members nationwide. This year, we have been offering a five-part Physical Therapy webinar series with Nicole Irizarry, DPT, as well as a five-part Fitness Education webinar series with Taylor Lewis, MA, CSCS. Our regular "Yoga for CF Health" classes were taught by Martha Modawell, Certified Yoga Instructor and mother of two daughters with CF, and Colleen Lewis, Certified Yoga Instructor and adult with CF. All our classes are held online via Zoom, and our instructors and class participants log in from throughout the United States and beyond. 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.org**.

CFRI's CF Wellness Initiative is supported through grants from Vertex Pharmaceuticals, Viatris, Translate Bio, Ionis Pharmaceuticals, and private donors.

Be the Change – CFRI Advocacy Efforts Address Issues Impacting the CF and Rare Disease Communities *By Siri Vaeth, MSW*

Through its *Many Voices ~ One Voice* Cystic Fibrosis Advocacy and Awareness Program, CFRI seeks to engage the CF community to raise awareness among the general public and legislative sectors about the burdens and complications of the disease, lack of cure, impact of its rare disease status, and the need for further support for CF research.

CFRI advocates seek to address issues that are detrimental to those impacted by the disease. Over the past six months, CFRI advocates have participated in virtual meetings with elected representatives and their aides at both the state and federal level. At the federal level we encourage increased financial support for the Food and Drug Administration (FDA) and National Institutes of Health (NIH).

On the state level, we have focused on the creation of Rare Disease Advisory Councils (RDACs), which provide the rare disease community with a formal platform and official voice at the state level to help advise state officials on policies and services that impact us. Currently, less than half the states in the US have an RDAC, and CFRI is a member of several coalitions working to advance these efforts.

CFRI also participates with several coalitions to address the proliferation of co-pay accumulator programs, that are increasingly embedded in private insurance plans and which create significant financial hardship for many members of our community. Co-pay accumulator programs do not allow payments from drug manufacturer discount cards to be applied toward a person's deductible and total out-of-pocket expenses. As a result, many individuals with CF and their families are shocked to find that they still have a large deductible to meet months into the calendar year. In essence, the deductible is paid twice.

Those who rely on specialty medications are most impacted by these policies. It has been found that one in four people with CF have delayed seeking care or skipped treatments because of costs related to insurance premium rates, deductibles, out-of-pocket expenses, and co-payments. CFRI is working with others to support legislation that will mandate that all payments for prescriptions be applied toward one's deductible and annual out-of-pocket total. This type of legislation has already passed in a dozen states and Puerto Rico, with broad bipartisan support.

CFRI has advocated for expanded telehealth services, which have been vitally important through the pandemic, but which have proven in many instances to increase adherence to clinic visits and alleviate stress for patients.

We are concerned by increasing efforts to expand pre-authorization requirements for prescriptions and treatments, and a growth in step therapy (or "fail first") requirements by insurance companies and pharmacy benefit managers. These programs deny patients access to the specific medications their physicians have prescribed, first forcing them to try and fail using less expensive medications. This interference with physician-prescribed treatment creates delays in necessary care that can have catastrophic consequences.

CFRI also continues to support improvements in state newborn screening programs. Cystic fibrosis impacts people of every race and ethnicity, and individuals from underrepresented groups are more likely to have rare mutations that will not be detected through many states' current screening panel. Awareness must be raised about the diversity of our community so as to address these disparities.

CFRI invites our community to engage with our advocacy efforts. Please email Stacie Reveles at sreveles@cfri.org for more information.

CFRI's Many Voices ~ One Voice CF Advocacy and Awareness Program is funded through educational grants from Vertex Pharmaceuticals, Genentech, Gilead Sciences, Ionis Pharmaceuticals, and AbbVie.

SAVE THE DATES!

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

Online Support Group for Adults with CF
Third Monday of Every Month
Go to www.cfri.org for information.
Participate by Zoom or phone
Support Groups sponsored by Chiesi USA, Genentech, Ionis Pharmaceuticals, Viatris, Translate Bio, Gilead Sciences, Vertex Pharmaceuticals, and private donors.

Support Group for CF Caregivers
Third Tuesday of Every Month
Go to www.cfri.org for information.
Participate by Zoom or phone

Online Support Group for Post-Transplant Adults with CF
Fourth Wednesday of Every Month
Go to www.cfri.org for information.
Participate by Zoom or phone
Sponsored by a private bequest

CF Spring Break
A virtual retreat for adults with CF, their friends and family
March 18 – 20, 2022

Embrace Mothers' Retreat
May 13 – 15, 2022
Vallombrosa Retreat Center (TBD)
Menlo Park, CA

CFRI's 35th National CF Education Conference ~ A Virtual Event
July 29 – July 31, 2022

CF Summer Retreat: For Adults with CF
August 2022
More details coming soon!

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

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Alicia Maciel, MBA
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Medify Air: Gary Tribolet
Paula Meier
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San Francisco Food Tours
San Francisco Giants
Santa Cruz Beach Boardwalk
Harini Seshadri
Chanda Seymour Design
Sheraton Hotel Redding
Soquel Vineyards
Swirl Works
Turtle Bay Exploration Park
Marc Uharriet
Walt Disney Family Museum
Windsor Vineyards
The Wynn Resort Las Vegas

Superwoman on a Lifesaving Mission: Anna Payne's Colon Cancer Crusade

By Siri Vaeth, MSW

She noticed that her GI symptoms were worse than usual, but that was nothing new. As a 34-year-old woman with cystic fibrosis, Anna Payne was accustomed to the unpredictable shifts in digestion related to her disease. But then she found a mass in her groin. A biopsy determined that it stemmed from an aggressive colon cancer that had rapidly metastacized. After a colonoscopy and a PET scan Anna learned that she had stage-4 colon cancer: a large mass in her colon had spread to her groin, lymph nodes, ovaries, and liver.

Colon cancer risks are extremely high for those with CF, with rates 5 to 10 times those

Continued on page 13

CFRI-Funded Researchers on the Forefront of Discovery

In addition to Xiaopeng Li, whose CFRI-funded research is described on the cover of this newsletter, the following researchers shared their work at CFRI's 2021 National CF Education Conference.

Regional Regulation of CFTR and Ionocyte Expression in Airways

Kenichi Okuda, MD, PhD
University of North Carolina, Chapel Hill, NC


Kenichi Okuda, MD, PhD

A full characterization of CFTR expression in normal conducting airways is lacking. Through our research, we aimed to identify cell types that contribute CFTR expression and function along the proximal-distal axis of normal human lung. Experiments were performed on human mainstem bronchial epithelial cells obtained from four healthy volunteers by bronchoscopic brush biopsy, and small airway epithelial cells obtained from three non-smoker lung transplant donors by microdissection. scRNA-seq identified mucous-secretory cells as dominating CFTR mRNA expression in normal human large and particularly small airways, followed by basal cells. Ionocytes expressed the highest CFTR levels per cell but were rare, while expression in ciliated cells was low. Single cell-based quantitative PCR (scqRT-PCR) and RNA in situ hybridization (scRNA-ISH) confirmed scRNA-seq data. RNA-ISH in normal lungs exhibited discordance between CFTR expression and ionocyte localization in the distal airways. CF lungs demonstrated distribution patterns of CFTR and iononcyte similar to normal controls. CFTR expression in mucous-secretory cells in normal human airways suggests the presence of a mechanism whereby airway mucin secretion is coupled to airway surface liquid volume regulation. In the small airway, the combined function of these mucous-secretory, CFTR-expressing cells are likely critical for preventing mucous obstruction and supporting normal MCC.

Personalized Phage-based Therapies

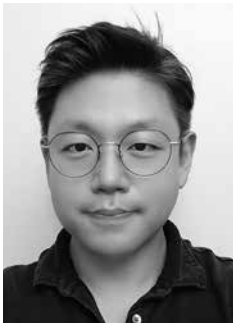
Forest Rohwer, PhD
San Diego State University, San Diego, CA


Forest Rohwer, PhD

Trikafta has dramatically changed the CF therapeutic landscape and most CF patients can look forward to improved quality of life. In this new era, CF lung infections have become even more personalized; complications range from opportunistic pathogen infection of legacy remodeled/scarred lung tissue to patients non-responsive to Trikafta. To address this changing landscape, we are simultaneously working on traditional phage therapy, as well as modified phage called tailocins, to create personalized treatments for CF lung infections. Our main targets are the multi-drug resistant, CF pathogens *Stenotrophomonas* spp. and *Achromobacter* spp. This presentation describes laboratory protocols for isolating and purifying these biologicals, as well as the progress in regulatory and clinical treatment protocols.

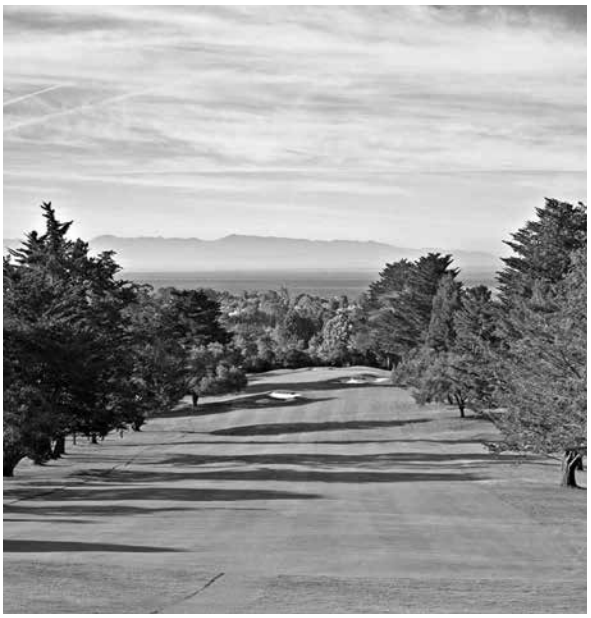
A Multi-'Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis

Keehoon Lee, PhD
The Pathogen and Microbiome Institute, Northern Arizona University, Flagstaff, AZ


Keehoon Lee, PhD

Several studies have shown that the possible connection between the upper and lower respiratory tract microbial composition of CF patients with CRS. In this study, we hypothesize that the specific microbial strains or encoded functions drive concurrent type I inflammation in upper and

Continued on page 12



37th Annual Golf Tournament Benefitting CFRI – Another Phenomenal Event!

On August 9, the world-famous Pasatiempo Golf Club – a "Top 100" course in Santa Cruz, California – hosted the 37th Annual Golf Tournament Benefitting CFRI in support of the search for a cystic fibrosis (CF) cure. This year's event raised a record $88,000 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 support of the many participants. 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 2022 will be announced soon!

Elizabeth Nash Memorial Fellowship Award:

Suzanne Fleiszig, PhD

Naren Kumar, PhD

New Horizons Award:

Matthew Porteus, MD, PhD

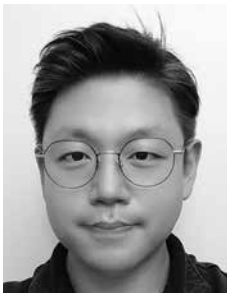
Sriram Vaidyanathan, PhD

Steven Jonas, MD, PhD

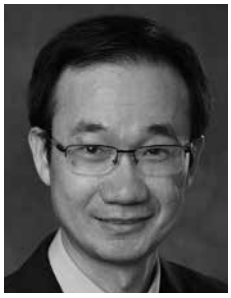
Steven Aller, PhD

Other CFRI-funded Researchers:

Emily Cope, PhD



Keehoon Lee, PhD



Xiaopeng Li, PhD

Kenichi Okuda, MD, PhD

Forest Rohwer, PhD

CFRI Funds Innovative CF Research

After an extensive vetting of a wide range of high quality research proposals by CFRI's Research Advisory Committee, the Board of Directors of CFRI voted to fund the following awards:

Elizabeth Nash Memorial Fellowship Award 2021:

Characterizing the intracellular diversification of Pseudomonas aeruginosa in chronic lung infections.
Suzanne Fleiszig, PhD, Principal Investigator;
Naren Kumar, PhD, Post-Doctoral Fellow; UC Berkeley

New Horizons Award 2021:

Identifying Biomaterials that Enable the Transplantation of Gene Corrected Airway Stem Cells to Treat Cystic Fibrosis.
Matthew Porteus, MD, PhD;
Sriram Vaidyanathan, PhD; Stanford University School of Medicine

Developing Nanotechnology-Enabled Gene Therapy Solutions to Correct CFTR Mutations in Airway Stem Cells: Toward a One-Time Cure for Cystic Fibrosis.
Steven Jonas, MD, PhD; The Regents of the University of California, Los Angeles

Role of CFTR Arginine-933 in Folding, Gating and Potentiator Drug Binding.
Steven Aller, PhD, The University of Alabama at Birmingham.

Others who conducted CFRI-funded research in 2021 include:

A Multi-'Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis.
Emily Cope, PhD, Principal Investigator;
Keehoon Lee, PhD, Post-Doctoral Fellow; Northern Arizona University

Targeting V-type ATPase in Human Small Airways for CF Lung Disease Treatment.
Xiaopeng Li, PhD; Michigan State University

Regional Regulation of CFTR and Ionocyte Expression in Airways.
Kenichi Okuda, MD, PhD; University of North Carolina Chapel Hill

Development of Tailocins Against Microbial Infections in Cystic Fibrosis Lungs.
Forest Rohwer, PhD; San Diego State University.

These promising research projects are made possible through generous contributions from the community. Thank you to all whose support is advancing innovative cystic fibrosis research.

CFRI-Funded Researchers on the Forefront of Discovery *Continued from page 11*

lower respiratory tracts of CF patients. To test the hypothesis, we used metagenomics and metatranscriptomics on paired upper and lower airway samples from healthy, non-CF-CRS, and CF-CRS patients with various comorbidities. Our metagenomics results showed a certain strain of *Staphylococcus aureus*, RF122, is associated with polyposis of the patients with CRS, but there was no particular association with the fungal microbiome and CRS or CF. The beta-diversity analyses also demonstrated significant differences between control, CRS, and CF respiratory microbiome. Furthermore, it presented no significant difference between the upper and lower respiratory microbiome which represents the possible microbial sharing between the two locations. The transcriptome analysis results demonstrated significantly different gene expressions between CF and CRS. The differentially expressed genes are mainly related to inflammatory and immune responses. The findings of this research helped us better understand the relationship between microbiome composition and host response, such as specific gene expressions and their functions in disease control.

The Hunger Is Real: Food Insecurity in the CF Community

By Georgia Brown

In 2019, researchers from the Milken Institute School of Public Health at George Washington University administered the "Cystic Fibrosis Health Insurance Survey." While this survey is conducted periodically to assess health status, access to medical care, and insurance plans for those in the CF community, the most recent survey included nine questions to assess medical cost burdens, food intake changes and the usage of programs designed to provide support. The additional questions were included in part to study the incidence of "Food Insecurity" (FI) in the CF community.

It is important to note that the survey was conducted with support from the Cystic Fibrosis Foundation, and prior to the release of Trikafta™ and before the COVID-19 pandemic.

In reviewing the results, the staggering find is that about 10,000 people with CF experienced FI within the 12 months preceding the survey. This is three times the national average! The survey results show that FI does not discriminate. For example, income is not a determinant of FI because 7% of those earning over $150,000 experienced FI. Even 9% of those who self reported as "living comfortably" experienced FI. While those dependent on governmental insurance programs faced FI at higher rates, so did nearly 20% of those on private insurance.

High treatment burden can lead to medical debt and those with medical debt experienced higher rates of FI. About one third of respondents under the age of 17 and over the age of 36 faced FI, as did nearly 40% of those between the ages of 18 and 35. Employment status does have an impact because nearly 60% of those on disability faced FI. However, of those working over 30 hours per week, 22% still faced FI.

It is important to fully understand what food insecurity in the CF community looks like. Imagine this: one out of every 5 respondents of this survey had trouble paying for food. About 20% lacked the funds to purchase the foods for the diet prescribed by their CF care team. Others ran out of food and lacked resources to purchase more. Many cut the size or even skipped meals because there was not enough food to

meet their nutritional needs. Sadly, some parents went hungry to ensure their child with CF did not. Yet, the rates for those who obtained help were relatively low compared to the indicated need.

What can we do? We can reduce the stigma of getting help. It is very likely that the incidence of food insecurity in the CF community has increased in the wake of COVID-19. The pandemic shed light on the increasing need for improving national programs, and increasing food banks and other resources to eliminate hunger in our country. We need to ensure that those in the CF community who face FI have information about – and access to – resources near their homes.

Superwoman on a Lifesaving Mission: Anna Payne's Colon Cancer Crusade

Continued from page 10

of the general population. For those who have received a transplant, the rate is 20 times that of the general population. "No one was thinking it was cancer because I am so young. I wish I had known how prevalent colon cancer is for those of us with CF. I would have had a colonoscopy years ago if I had known."

Anna now understands that many of her symptoms mimicked her CF-related GI symptoms. It was easy to blame the stomachaches, bloating and variable bowel habits on cystic fibrosis. But in a day, her world changed completely. At 34, Anna had her first port implanted to begin chemo. "Ironic, right? I always felt so fortunate that I managed to avoid having a port for my CF. Since Trikafta I have felt better than I have in years, and here I am with a port for my chemo."

Anna is currently in a battle for her life. Months ago she was extremely active, working for Bucks County in Pennsylvania, serving as an elected Township Supervisor as well as on Pennsylvania's Rare Disease Advisory Council. Now she is in the midst of chemotherapy treatments: her hair is gone, she is nauseous and deeply fatigued. But despite her personal challenges, Anna has donned a metaphorical cape to help save the lives of others, by advocating for changes in the recommended age for people with CF to have their first colonoscopy. In sharing her story, she hopes that others will not delay this vital screening.

Because colon cancer is now increasingly diagnosed among younger people, the recommended age for the general public to have a first screening colonoscopy has been lowered from 50 to 45 years. Currently, the recommended age for a first colonoscopy for those with CF is 40. "If the age for the general public has dropped to 45, why hasn't the aged dropped for those with CF, when it is known that we have this incredibly high risk? I wish it would be changed to 30 years for the CF community. Even 35 would have been too late for me."

As for those who are squeamish about having a colonoscopy, Anna says, "If you think a colonoscopy is hard, you've got it all wrong. What's hard is battling aggressive cancer. Please learn from my experience and get screened."

A video podcast with Anna is available on CFRI's YouTube channel.

CFRI Is Your Partner in Living

- **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.
- **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.
- **HOLD YOUR OWN VIRTUAL EVENT:** Cocktails for a cure, yoga, corn hole, 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!
- **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:** Benefits can include increased income, substantial tax savings, the opportunity to meet your philanthropic goals, and the satisfaction of making a very significant gift to CRFI 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 Programs and Advocacy Associate: 650.665.7586 or sreveles@cfri.org.

From Nutrition and Gene Therapy to Diversity in Our CF Community: *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, conference presentations, ototoxicity, and more. In addition, there are videos from our Diversity and Inclusion Initiative. Many of the episodes are available with Spanish and Hindi subtitles, as well as captioning in English for the hearing impaired. 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 Viatris.*

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.

2021 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.

Dave Stuckert Memorial Volunteer of the Year Award — Marina Gonzales

Marina is the sister of an adult with CF and has been an active volunteer with CFRI for over a decade in a variety of capacities. She serves on CFRI's CF Summer Retreat Committee, participates in CFRI advocacy events, and serves on CFRI's Gala Committee. Marina is a team player who brings warmth, good humor and humility to her volunteer work. She is beloved by all who know her and have the honor of working with her.

Professional of the Year Award — Yelizaveta Sher, MD, FACLP

Dr. Sher is a member of Stanford's Psychosomatic Medicine Faculty and serves as a Mental Health Coordinator for the Adult Cystic Fibrosis Clinic at Stanford. She has authored/co-authored many articles on the psychiatrist's role in transplant and co-edited three books addressing CF, transplant and COVID-19. Dr. Sher has volunteered with CFRI for many years, presenting on CF and mental health issues while promoting CFRI's counseling and Quality of Life services. Since March 2020, Dr. Sher has shared her time and expertise to moderate a COVID-19 CF support group attended by CF community members from across the country. She is a true community hero.

CFRI Partners in Living Award in Memory of Anabel Stenzel — Jacob Fraker, MSW

Jacob is a Legislative Aide for California State Senator Susan Eggman, where he serves as the Legislative Consultant for the CA Legislative LGBTQIA+ Caucus. An adult with cystic fibrosis, Jacob initially volunteered with CFRI, then served as CFRI's legislative analyst, then conducted his Master's internship with CFRI. In his work with Senator Eggman, he advocates for the CF, rare disease LGBTQIA+ communities. Jacob has furthered CF and rare disease awareness at the state level while consistently supporting CFRI, including serving on CFRI's CF Adult Advisory Committee. Jacob embodies the spirit of this award and Anabel Stenzel's memory.

Paul M. Quinton Cystic Fibrosis Research Legacy Award — Jonathan Widdicombe, PhD

Dr. Widdicombe is Professor Emeritus, Department of Physiology and Membrane Biology, at the University of California at Davis. Over twenty years ago, Dr. Widdicombe and his team determined that airway epithelial cultures obtained from CF patients were unable to secrete chloride ions. Much of his research focused on determining how a defect in chloride ion transport results in CF pathology. Dr. Widdicombe served for many years on CFRI's Research Advisory Committee, where he established and directed the Elizabeth Nash Memorial Fellowship Program. Dr. Widdicombe's research has had – and continues to have – an enduring impact upon the field of cystic fibrosis.

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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, Genentech, Gilead Sciences, AbbVie, Chiesi USA, Viatris,** *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 A Breath of Fresh Air Virtual Gala A Big Success!

CFRI's annual gala, held virtually on October 16, 2021, brought together a community united in the search for a cure for cystic fibrosis. A lively pre-gala event was attended by community members across the country, as Emily Schaller, CF warrior and fearless leader of the Rock CF Foundation, led us in preparing a signature event cocktail. The official gala program launched with a warm welcome from Olympic champion Kristy Yamaguchi, while our emcee, Emmy-award winning Chris Chmura of NBC Bay Area, guided us through a fast-paced but moving program in which the experiences of dozens of CF community members were shared. We honored the phenomenal Jennifer Taylor-Cousar, MD, MSCS, of National Jewish Health, as our 2021 CF Champion. Warm thanks to all who participated in the production of our gala – individuals with CF, parents of those with CF, care providers, and researchers. The auction led to spirited bidding until the last minute, while donations to our Bid for a Cure grew. By the end of the evening, over $133,000 was raised to support CFRI's research, education and support programs, and $45,000 of this total is being matched dollar-for-dollar by members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle and designated for our CF research awards.

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!*

Please see page 10 *for a complete list of our sponsors and in-kind and auction donors.*

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