37th National Cystic Fibrosis Education Conference

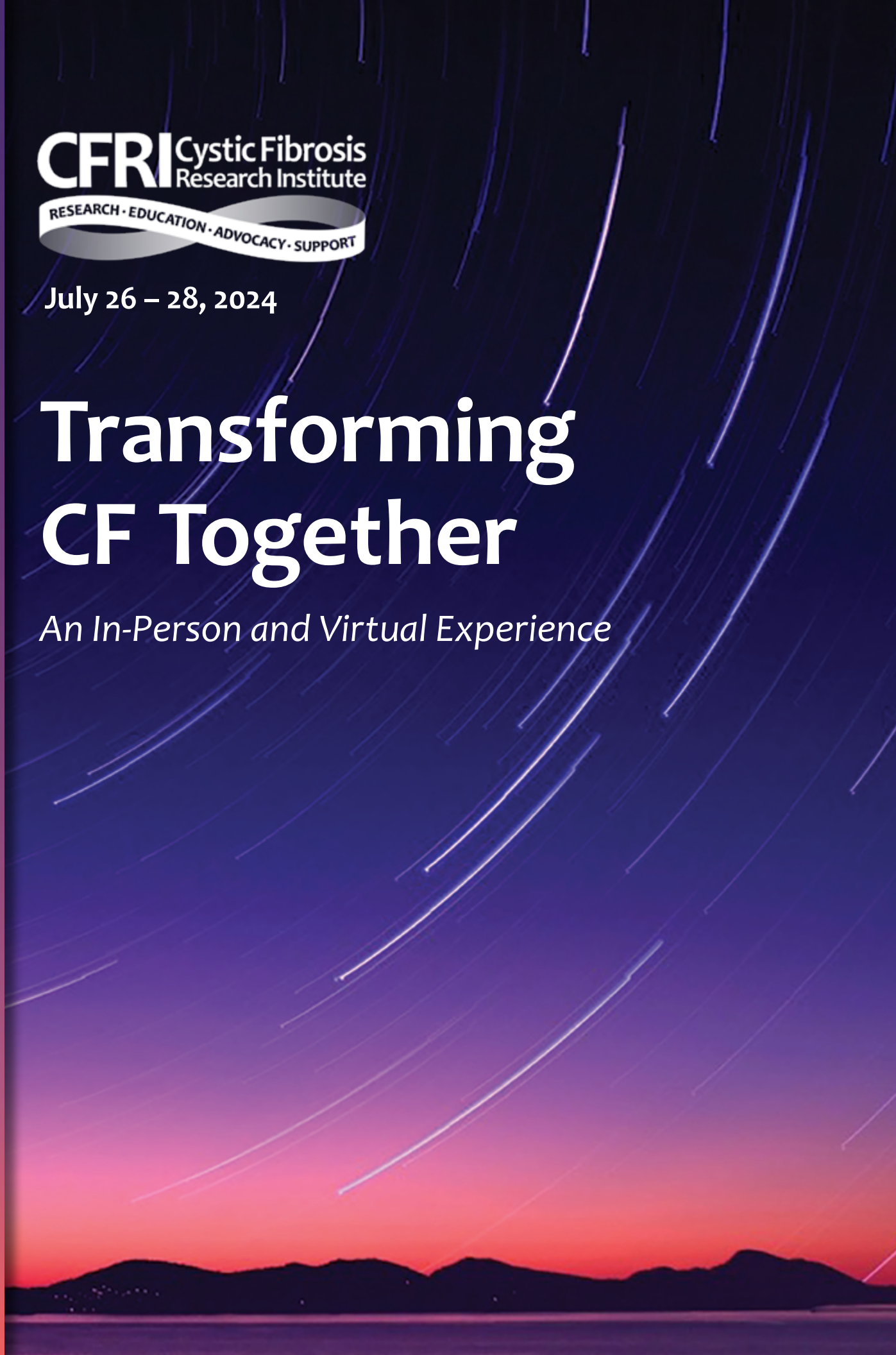
CFRI Cystic Fibrosis Research Institute

RESEARCH · EDUCATION · ADVOCACY · SUPPORT

July 26 – 28, 2024

Transforming CF Together

An In-Person and Virtual Experience



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Greetings

Congress of the United States
House of Representatives
Washington, D. C. 20515

Anna G. Eshoo
Sixteenth District
California

July 26, 2024

Dear Friends,

It is my pleasure to welcome you to California's 16th Congressional District and to CFRI's 37th National Cystic Fibrosis Education Conference, *"Transforming CF Together."*

Cystic fibrosis is a life-threatening genetic disease affecting the respiratory, digestive and reproductive systems. Since 1975, the Cystic Fibrosis Research Institute has funded innovative cystic fibrosis research, raised public awareness of the disease, and provided vital education and support services to the CF community.

This weekend's conference continues CFRI's goal of offering community members the opportunity to learn from leaders in the field, while providing patients, families, medical caregivers, and cystic fibrosis-related technology and pharmaceutical representatives the opportunity to share and enhance understanding and treatment of the disease.

On behalf of the people of California's 16th Congressional District, I thank the Cystic Fibrosis Research Institute for its superb work, and I offer everyone my best wishes for the success of this year's conference.

I'm proud to be your partner in Congress to advance research to find a cure for cystic fibrosis.

Warm regards,

Anna G. Eshoo
Member of Congress

Welcome

Dear Friends,

Welcome to CFRI's 37th National Cystic Fibrosis Education Conference, *Transforming CF Together*. We are delighted to offer this year's conference as a hybrid event, that will offer the opportunity for all members of our community to come together – whether in person or virtually.

Due to the efforts of individuals with cystic fibrosis and their families, researchers, CF-related organizations, pharmaceutical companies, and clinicians – we are advancing therapies and moving closer to a cure. Exciting progress continues in the field of CF, and we are inspired and immensely proud of CFRI's role in these advances.

Our 2024 conference provides the opportunity to hear from over 20 experts in the field of cystic fibrosis, addressing mRNA therapies, gene editing, phage therapy, medical trauma, cancer, reproductive health, equity and inclusion, and much more. We are extremely grateful to all of our presenters who are generously sharing their time and expertise.

Our annual conference also provides us with the opportunity to celebrate heroes in the field. On Saturday evening we honor our 2024 outstanding volunteer, professional, and researcher of the year, as well as an inspirational adult with cystic fibrosis. Please join us at our awards celebration, and for those of you attending in person, this will be followed by a lively dance party.

We thank our generous sponsors, whose support makes this conference possible. Many representatives are here, and we sincerely hope that you will introduce yourselves to them and to all of our exhibitors. They have been key partners in much of the progress that we celebrate.

CFRI remains steadfast in its mission to be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support. Our vision is to find a cure for cystic fibrosis while enhancing quality of life for the CF community.

CFRI is your partner in living, today, and into the future. Thank you for being a part of this caring and engaged community.

Warm regards,

Bill Hult
President, CFRI Board of Directors

Siri Vaeth, MSW
Executive Director, CFRI

Conference Schedule — *Transforming CF Together*

All times listed in Pacific Times. Presentation times may vary slightly.

Friday, July 26, 2024 (In Person and Virtual)

NOTE: Friday Morning and Afternoon Speakers are Presenting Their CFRI-Supported Research.

8:00 am – 8:45 am ***Sequoia Room***	**Continental Breakfast**
8:50 am – 9:00 am ***Acacia Room***	**Welcome and Opening Remarks** — Siri Vaeth, *MSW, CFRI Executive Director* **Introduction of Research Presentation Emcee** — Julie Desch, *MD, Research Advisory Committee Chair*
9:00 am – 9:45 am ***Acacia Room***	**Role of IRBIT in Ion Transport Trafficking in Healthy and Cystic Fibrosis Intestine** — Zachary Sellers, MD, *PhD*
9:55 am – 10:40 am ***Acacia Room***	**Improving CF Airway Mucociliary Clearance: Toward Transition from Animals to Humans** — Nam Soo Joo, *PhD*
10:40 am – 11:05 am	**Break**
11:05 am – 11:50 am ***Acacia Room***	**Pf Bacteriophage is Associated with Antibiotic Tolerance and Poor Outcomes in Patients with Cystic Fibrosis** — Paul Bollyky, *MD, PhD*
11:50 am – 12:45 pm ***Sequoia Room***	**Lunch Break**
12:45 pm – 1:30 pm ***Acacia Room***	**A Hybrid Gene Correction Strategy for Cystic Fibrosis** — Anais Amaya, PhD; Matthew Porteus, *MD, PhD*
1:50 pm – 2:35 pm ***Acacia Room***	**Triple Whammy: Targeting Recalcitrant CF Pathogens with Phages, Antibiotics, and Small Molecule Adjuvants** — Katrine Whiteson, *PhD*
2:45 pm – 3:30 pm ***Acacia Room***	**CFTR High Expresser Cells Are pH-Sensing Neuropods** — Nadia Ameen, *MBBS*
3:30 pm – 3:45 pm	**Break**
3:45 pm – 5:15 pm ***Peninsula 1 – 4***	**Support Groups** (In Person and Virtual) — *Adults with CF* — *Parents/Caregivers of Children with CF* — *Parents/Partners of Adults with CF* — *Adults Post Transplant*
3:45 pm – 4:30 pm ***Acacia Room***	**Molecular Regulatory Pathways Associated with the Distinct Identity of Large and Small Airway Epithelia in Humans** — Kenichi Okuda, *MD, PhD*
4:35 pm – 5:20 pm ***Acacia Room***	**CFTR mRNA Delivery to Cystic Fibrosis Airways** — Beate Illek, *PhD*
5:30 pm – 6:15 pm ***Dockside Room***	**In-Person Reception**
6:15 pm – 7:00 pm ***Dockside Room***	**The Power of Passion** — Nicholas Kelly, *MS, RD, LD* (Inspirational Presentation)
7:00 pm – 8:15 pm	**Break**
8:15 pm – 9:15 pm ***Acacia Room***	**Book Reading: *Love, Courage and Miracles*** — Robin Modlin, MA

Saturday, July 27, 2024 (In Person and Virtual)

7:30 am – 8:30 am ***Sequoia Room***	**Continental Breakfast**
8:45 am – 9:00 am ***Acacia Room***	**Welcome and Opening Remarks** — Siri Vaeth, *MSW, CFRI Executive Director* — Introduce Emcee, Rohini McKee
9:00 am – 9:55 am ***Acacia Room***	**Phage Therapy for CF-Accociated Infections** — Benjamin Chan, *PhD*
10:05 am – 11:00 am ***Acacia Room***	**Modeling Epithelial Immune Cell Interactions in Cystic Fibrosis** — Amy Ryan, *PhD*
11:00 am – 11:15 am	**Break**
11:15 am – 12:10 pm ***Acacia Room***	**Aging in the New Age of Cystic Fibrosis** — Richard Moss, *MD*
12:10 pm – 1:15 pm ***Sequoia Room***	**Lunch Break**
1:15 pm – 2:10 pm ***Acacia Room***	**Culture Shift: CF Lung Infections in the Modulator Era** — Lucas Hoffman, *MD, PhD*
2:20 pm – 3:15 pm ***Acacia Room***	**Sexual and Reproductive Health in CF** — Natalie E. West, *MD, MHS*
3:15 pm – 3:30 pm	**Break**
3:30 pm – 4:25 pm ***Acacia Room***	**Panel: Coping with Cancer and CF** — Elyse Elconin-Goldberg, *MA*; Thomas Horal; Colleen Lewis; Christine Nash, *MBA*; Moderated by Jean Hanley, *MD*
4:25 pm – 5:30 pm	**Exhibitor Hall / Break**
5:30 pm – 6:00 pm ***Sequoia Room***	**Dinner Buffet** (In Person)
6:00 pm – 7:15 pm ***Sequoia Room***	**CFRI Awards Celebration with Special Guests**
7:30 pm – 9:30 pm ***Dockside Room***	**Dance Party** (In Person)

Sunday, July 28, 2024 (In Person and Virtual)

8:00 am – 9:00 am ***Sequoia Room***	**Continental Breakfast**
9:00 am – 9:10 am ***Acacia Room***	**CFRI Overview** — Siri Vaeth, *MSW, CFRI Executive Director*
9:10 am – 10:05 am ***Acacia Room***	**Panel: Advocacy, Access and Health Equity in Cystic Fibrosis** — Rachel Alder, Jaelyn Cooper, *MHA*; Alicia Maciel, *MBA*; Abhijit Tirumala; Moderated by Kimberly Morse, *MSW, LCSW*
10:15 am – 11:05 am ***Acacia Room***	**Exploring Nucleic Acid Based Approaches to Treat People with Cystic Fibrosis** — Jennifer Taylor-Cousar, *MD, MSCS*
11:05 am – 11:20 am	**Break**
11:20 am – 12:15 pm ***Acacia Room***	**Strategies to Address Medical Trauma** — Samantha Johnson, *MA, CCLS*; Kate Yablonsky, *LCSW*
12:20 pm – 1:10 pm ***Acacia Room***	**Living Proof: Nearly Seventy Years with Cystic Fibrosis** — Luanne McKinnon, *PhD* (Inspirational Presentation)
1:10 pm – 1:15 pm ***Acacia Room***	**Closing Remarks** — Siri Vaeth, *MSW, CFRI Executive Director*

Tips for Navigating the Virtual Conference

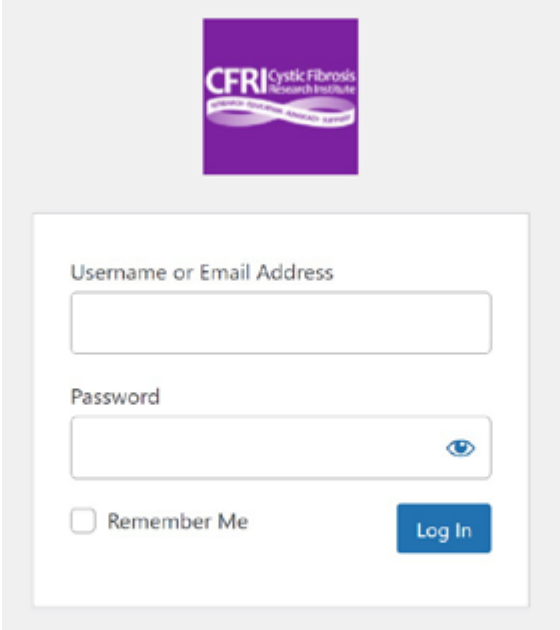
Virtual Attendee Guide

Those attending the conference virtually can access all the presentations and content from most computers and mobile devices such as laptops, desktops, and handheld tablets.

Login Screen

To access the conference you must first login with your conference credentials (pictured right). This will be the email that you registered with and conference password.

Once logged in, click on the button: "You are logged in!"



Main Lobby

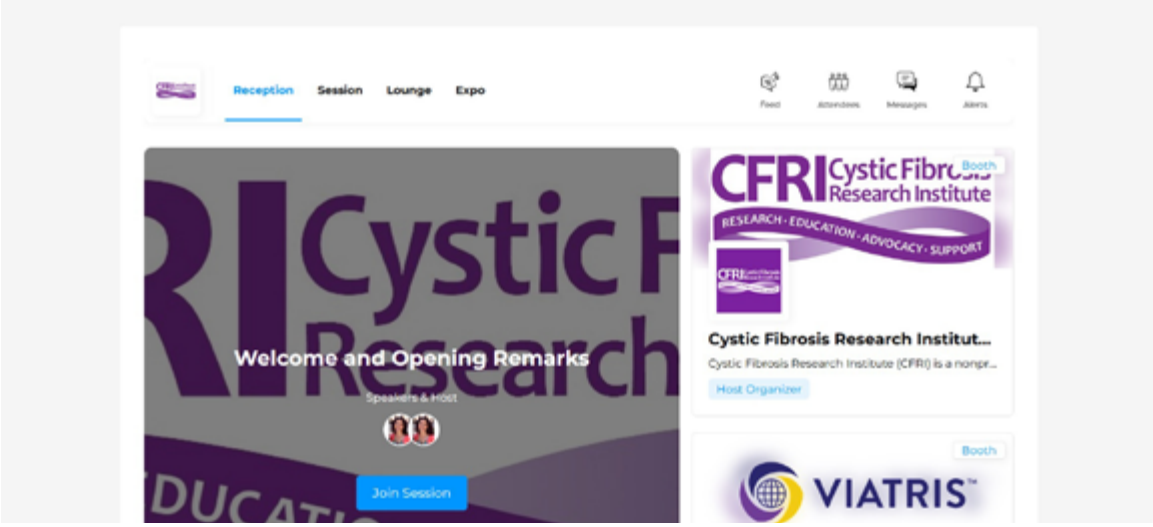
In the main lobby, attendees may **use the menu at the top of the screen** to access the Auditorium (watch streamed sessions), Exhibit Hall (visit the virtual booths), and the Lounge (to Video Chat with other attendees). From the menu attendees can also watch the welcome video, view the Conference Agenda, and if help is needed, the CFRI Support and Tech Support desks are open all weekend.

Tips for Navigating the Virtual Conference

Reception Area

The Reception Area can also be accessed from the menu at the top of the screen. This is where you will see what session is in progress, or soon to begin. As you enter the Reception Area, please allow a few moments for the content to load.

CFRI – Cystic Fibrosis Research Institute Transforming CF Together Education Conference



Lounge

In the Lounge, you can video chat with other attendees on shared topics of interest. Choose your topic and click on **Join Table**.

Header Menu Definitions

Reception: Display the upcoming session.

Sessions: View a list of sessions.

Lounge: Access a list of tables with topics and connect with your peers in a small group video setting.

Expo: Visit the Exhibitor Hall with virtual booths of participating vendors.

Menu on Right Definitions

Feed: A global chat for all attendees to post comments.

Attendees: Attendees logged into the conference. Attendees can connect and chat 1:1.

Messages: Direct messages to an attendee.

Alerts: These are Push Notifications to all attendees.

Hygiene Guidelines for All Attendees

CFRI is dedicated to minimizing cross-infection risk for all in attendance. **All in-person conference attendees – whether or not they have CF – must follow the hygiene guidelines listed below so as to limit the risk of cross-infection.** These guidelines have been developed in collaboration with our medical advisors and apply to everyone, including those without CF.

1. CFRI requires all in-person attendees to attest that they have received all COVID-19 vaccines for which they are eligible and that they will take a COVID test within 24 hours prior to arriving at the conference to confirm that they are negative for COVID-19.
2. Everyone in attendance is encouraged to wear a mask at all indoors events. Bacteria in sputum may last for hours and may be passed to others. For those preferring to wear a mask, N95 and surgical masks will be provided.
3. Everyone in attendance is required to wear a nametag at all times, which documents that you are registered to attend. If you see someone without a nametag, please let a CFRI staff member know.
4. Please refrain from shaking hands, hugging or touching other people to avoid spreading germs.
5. If you do not feel well, you must leave the conference, regardless of whether it is COVID-19, a cold, virus or the flu. The conference is available to view live on our virtual platform, and recordings will be available to view online after the event.
6. For those with CF, try to maintain the "6-foot" rule to minimize cross-infection risk.
7. All participants with CF were required to have completed a sputum culture after June 12, 2024.
8. Individuals with CF cannot attend the conference unless they have been approved to do so by their medical team and CFRI.
9. Each person with CF must have submitted a medical release signed by their CF physician indicating they:
   - have never tested positive for an organism belonging to *Burkholderia cepacia complex* (Bcc);
   - have not cultured *Methicillin Resistant Staphylococcus aureus* (MRSA) within the past 12 months;
   - have not had a positive culture for *Nontuberculous mycobacteria* (NTM) in the past 12 months;
   - do not currently culture positive for any pandrug-resistant (PDR) bacteria (bacterial isolates non-susceptible to all agents in all antimicrobial categories) or extensively drug resistant (XDR) bacteria that remain susceptible to only one category of antimicrobials (does not apply to XDR isolates remaining susceptible to two or more categories of antimicrobials). Although negative sputum cultures do not eliminate risk, they may reduce risk of pathogen transmission.
10. Please cover your mouth with a tissue when coughing and immediately dispose of the used tissue. Do not dispose of sputum in toilets or sinks. Always disinfect your hands after coughing.
11. Do not share cell phones, pens, glasses, soda cans, plates, or eating utensils with anyone.
12. Those with CF will be served their meals by hotel personnel or CFRI volunteers. If you have CF, please refrain from touching any serving utensils. When food is being served, avoid passing food, glasses, pitchers, etc. to others.
13. Disinfect your hands before eating. Hand sanitizer will be provided.

Sponsors and Exhibitors

CFRI Recognizes Our Generous Sponsors and Exhibitors For Their Support of the 37th National CF Education Conference

Transformative Sponsor — *Vertex Pharmaceuticals*

Premiere Sponsor — *Viatris*

Platinum Sponsors — *AbbVie; Gilead Sciences; Boomer Esiason Foundation*

Gold Exhibitors — *Nestlé Health Science; ReCode Therapeutics*

Silver Exhibitor — *Cystetic Medicines*

Bronze Exhibitors — *Alcresta Therapeutics; Digestive Care, Inc.; 4DMT; Natera; Sionna Therapeutics*

Organizational Exhibitors — *Cystic Fibrosis Engagement Network (CFEN); Emily's Entourage*

Supporter — *Prodigy Press, Inc.*

List current as of 07/10/2024. Updates to list available in digital program.

Grand Bay Hotel San Francisco

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Redwood City, CA 94065

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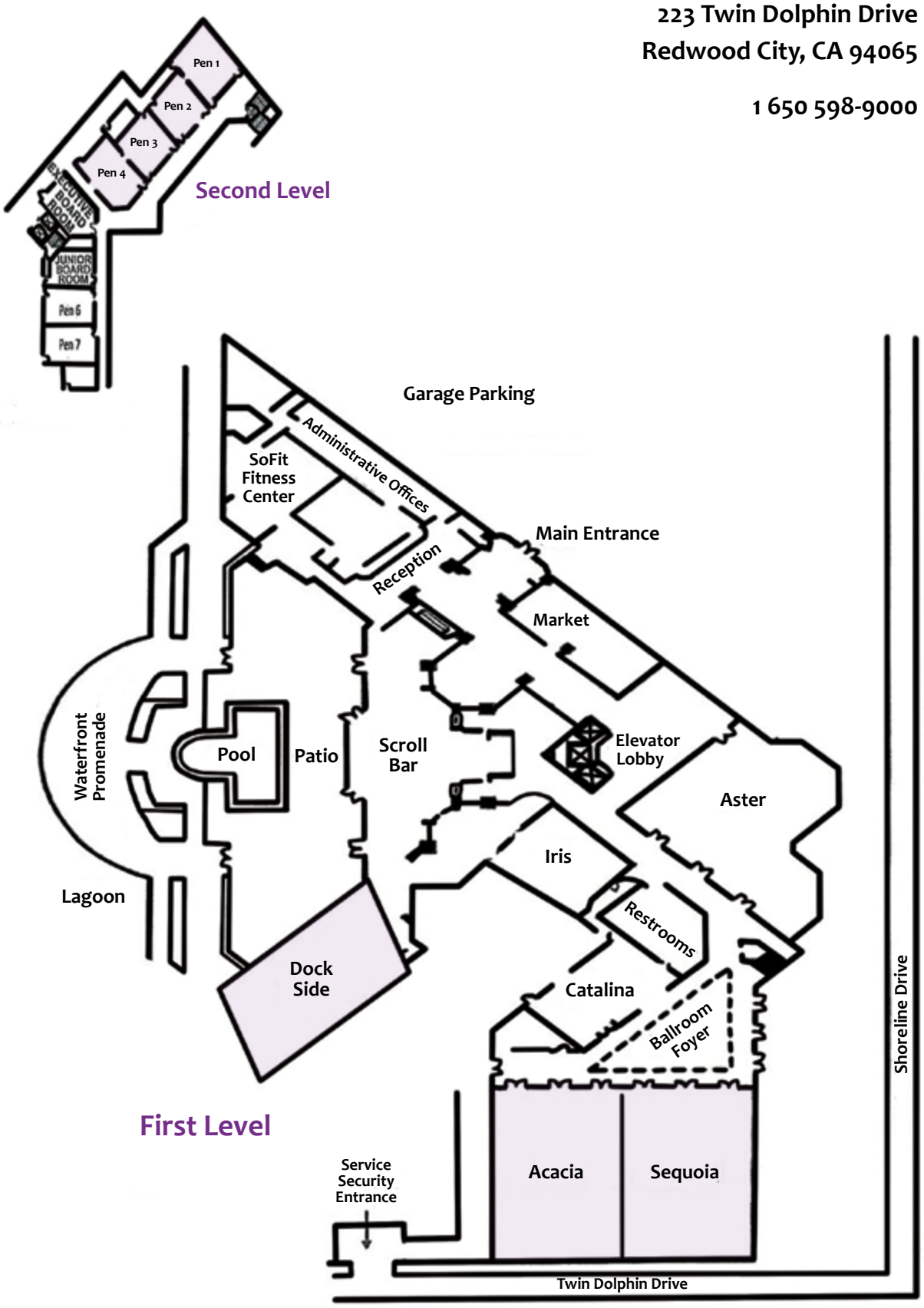


CFRI Leadership and Conference Emcees

Bill Hult — CFRI Board President

Bill Hult joined CFRI's Board of Directors in 2004 and currently serves as President. Bill's many years of nonprofit experience began in 1991 with service on the Meriwest Credit Union Supervisory Committee. He was a Director on the Board of Big Brothers Big Sisters of Santa Clara County and a founder of Big Brothers Big Sisters of the Bay Area. Bill served for over a decade on the West Valley/Mission College Citizens Bond Oversight Committee as well as the Responsible Landlord Engagement Initiative, sponsored by Catholic Charities. Bill is retired from IBM. He and his wife, Vicci, live in the Santa Cruz Mountains, and enjoy their five grandchildren, gardening, cycling, and hiking.

Siri Vaeth, MSW — CFRI Executive Director

Siri Vaeth has been CFRI's executive director since 2018, but her involvement with the organization began soon after her daughter Tess' diagnosis with CF in 1995. As a CFRI volunteer, she raised funds, chaired the Newsletter Committee, and served for 10 years on the Board of Directors. She joined CFRI's staff in late 2013. Siri has a BA in Politics from UC Santa Cruz, and a Master's in Social Welfare from UC Berkeley. She brings many years of nonprofit experience to CFRI, previously serving as executive director of Big Brothers Big Sisters of Santa Cruz County, nonprofit grant writer, United Way campaign associate, and social worker with Migrant Head Start. In addition to serving with several patient advocacy coalitions, Siri is a proud member of the American Thoracic Society's Public Advisory Roundtable. Siri's daughter Tess is now 29, and her son Dylan is 25. She lives in Santa Cruz, California.

Julie Desch, MD — Friday Emcee

At 63 years of age, Julie wakes up every morning amazed and grateful to be alive and healthy, breathing with her native lungs despite two copies of F508del. Her interest in CF research led her to Stanford Medical School, where she worked with Dr. Jeffrey Wine in the Cystic Fibrosis Research Laboratory as she pursued her medical degree. She then completed a residency and two fellowships in Anatomic Pathology at Stanford. After training, she worked at Kaiser Hospital in San Francisco, California as a surgical and skin pathologist. After retiring to take better care of herself and to be a full-time mom, she became a certified personal trainer and wellness coach for children and adults with CF before pursuing teacher training in Mindfulness-Based Stress Reduction, followed by a two-year Mindfulness Teacher Training Certification program, and then coach training in Unified Mindfulness. She has taught meditation online for the last eight years. Julie serves on the Board of Directors of CFRI and serves as Chair of CFRI's Research Advisory Committee.

Rohini McKee — Saturday Emcee

Rohini is the mother of two active and fun kids! Ria is 8 and living with cystic fibrosis and Rory is 2 and always making sure his sister is coughing during her treatments and taking her medicine. Rohini and Ria have been involved with CFRI since Ria was 1. On the weekends, you can find Rohini, her husband Richard, and their two kids searching for the best berries at a local farmers market, going on hikes, and watching Bluey.

Rohini is a Partner at Catalyst:Ed, an education organization that focuses on improving outcomes for America's children and youth by strengthening the schools and nonprofits that serve them. She also serves on Stanford's CF Family Advisory Council and the Boys and Girls Club of the Peninsula Programs Committee.

Speaker Profiles

*** denotes CFRI-funded researchers**

Anais K. Amaya, PhD *

Stanford University / Palo Alto, CA

Dr. Anais Amaya is a Postdoctoral Research Fellow in the Department of Pediatrics at Stanford University, where she specializes in gene therapy and genome editing with a particular emphasis on treating inherited diseases such as cystic fibrosis. She obtained her PhD in Medicine from the University of Sydney in Australia, during which she developed cutting-edge therapies for pediatric genetic liver diseases using CRISPR/Cas9 and adeno-associated viral (AAV) vectors. Her research includes developing a human-specific strategy to correct mutations in the OTC gene for the treatment of a Urea Cycle Disorder.

Born and raised in Venezuela, Dr. Amaya began her academic journey at Universidad Simón Bolívar, graduating Summa Cum Laude with a degree in Biology. She then pursued advanced studies in Molecular Biology and Biomedicine at Lund University in Sweden. Throughout her career, she has been honored with numerous awards, such as the Swedish Institute Scholarship, the Panos Ioannou Young Investigator Award from the Australasian Gene and Cell Therapy Society, and the Stanford Maternal & Child Health Research Institute Postdoctoral Award. Anais is committed to advancing her field by focusing on the development of safer and more effective delivery vectors for gene therapy, aspiring to contribute to the creation of curative treatments.

Nadia Ameen, MBBS *

Yale School of Medicine / New Haven, CT

Dr. Ameen is a physician-scientist, and Professor of Pediatrics (Gastro-enterology) and Molecular Physiology at Yale School of Medicine. She has successfully led an NIH-funded laboratory that has been investigating CFTR and its regulation by tralic in the intestine for over 25 years. She was the first to localize CFTR in endosomes in the subapical compartment of intestinal epithelial cells in the native intestine. This led to elucidation of brush border tralicking as a major mechanism regulating CFTR and fluid secretion in the intestine. She elucidated the role of myosin motors in CFTR tralic in the intestine using KO mouse models and showed the importance of myosins and CFTR in intestinal diseases. She made several notable ground-breaking discoveries in the CFTR field including the discovery of a rare subpopulation of enterocytes in the rat and human small intestine that she named CFTR High Expresser cells (CHEs). scRNAseq advances have identified gene signatures of CHEs that point to their importance in CF pathophysiology in the intestine.

Outside of her research, she has led mentorship programs for under-represented students, post docs, and faculty, and recently completed a role as Chair of the Dean's Diversity and Inclusion Committee for Faculty at the Yale School of Medicine.

Paul Bollyky, MD, PhD *

Stanford University / Palo Alto, CA

Paul Bollyky (pronounced "boy-key") is an Associate Professor and an infectious Disease physician at Stanford University. Paul is originally from Stanford, Connecticut. He received his DPhil at the University of Oxford, and his MD at Harvard Medical School. He completed his residency training at Brigham and Women's Hospital and then his Fellowship Training in infectious Diseases and Immunology at the University of Washington in Seattle. Paul joined the Stanford University Medical School faculty in 2013. He

Speaker Profiles

is an Associate Professor and has appointments in Immunology, Microbiology & Immunology, as well as Infectious Diseases.

At Stanford, his lab studies trans-kingdom interactions between bacteriophages, bacteria, and their human hosts. His team is interested in understanding how these interactions contribute to health and disease and in using bacteriophages to treat chronic infections in cystic fibrosis.

Benjamin Chan, PhD *

Yale University / New Haven, CT

Benjamin Chan, PhD, is an Associate Research Scientist in the department of Ecology and Evolutionary Biology at Yale University in the Laboratory of Professor Paul Turner. He is known for his work in phage therapy exploiting genetic trade-offs to treat antibiotic resistant bacterial infections. His research involves the development and creation of Virulence Targeting Antibiotics (VTA's) and Resistance Targeting Antibiotics (RTA's) for the treatment of bacterial infections refractory to traditional antibiotic therapy. His work spans the entire 'bench to bedside' spectrum and he has successfully isolated, characterized, and used bacteriophage-based V/RTA's to treat several infections (with the permission of the FDA). His research was featured in the Netflix series, "Follow This," as well as in documentaries produced by Vice, Freethink, and BBC One, and has reinvigorated phage therapy in Western medicine.

Lucas Hoffman, MD, PhD

University of Washington / Seattle, WA

Dr. Hoffman is a Professor of Pediatrics and Adjunct Professor of Microbiology at the University of Washington, Seattle and Seattle Children's Hospital. Dr. Hoffman received his medical and graduate degrees at the University of California, San Francisco. He completed both his residency in Pediatrics and his postdoctoral fellowship at the University of Washington (UW), and he joined the UW faculty in 2004. He currently serves as Vice Chair of Research for the Department of Pediatrics and Director of Scholarship for the Division of Pediatric Pulmonary and Sleep Medicine at UW. He also directs a National Resource Center, the Center for CF Research, supported by the CF Foundation that provides services, advice, and expertise in CF microbiology for researchers and industry partners. Dr. Hoffman's research and clinical efforts for the past 20 years have focused on the microbiology of people with CF, both in the respiratory and gastrointestinal tract.

Beate Illek, PhD *

University of California San Diego / San Diego, CA

Dr. Illek is an Adjunct Professor at the Department of Pediatrics at the School of Medicine at UC San Diego and the recipient of a 2024 CFRI Special Circumstance Research Award. Beginning with her foundational work in electrophysiology and epithelial cell biology at the University of Berlin, she has devoted her research career to study the cellular mechanisms underlying CF and developing potential treatments. Her early studies on ion transport in amphibian lungs laid the groundwork for her future endeavors. Transitioning to the United States with a scholarship from the German Academic Exchange Service, she immersed herself in the vibrant CF research community in the Bay Area, where she made groundbreaking contributions. Dr. Illek's work elucidating CFTR's role as a chloride and bicarbonate channel, along with her pioneering efforts in developing CFTR modulators, significantly advanced our

Speaker Profiles

understanding and treatment of CF. Her ability to translate basic research into clinical applications was demonstrated through her proof-of-principle work on the G551D gating mutation, which paved the way for the development and FDA approval of the first CFTR modulator drug. This achievement underscores the importance of bridging the gap between bench science and patient care. Dr. Illek's tenure as a Full Adjunct Professor at the Department of Pediatrics at UCSF Benioff Children's Hospital further solidified her impact on CF research. Not only did she continue her investigations into CFTR function and modulator responses, but she also contributed to the worldwide distribution of critical cell lines essential for CF research.

Her dedication to mentoring and education is evident through initiatives like the Cystic Fibrosis Summer Research Program, which has provided invaluable opportunities for aspiring researchers, including those living with CF. Over the years, she has trained numerous individuals in electrophysiological techniques, nurturing the next generation of CF scientists. Recognition of Dr. Illek's contributions to the CF community, such as the CFRI Professional of the Year Award and the Paul Quinton Research Legacy Award, underscores her tireless commitment to advancing our understanding of CF, seeking a cure, and inspiring others to join the fight against this disease. Her ongoing exploration of novel delivery formulations for nucleotide-based therapies represents yet another promising avenue in CF research, offering hope for improved treatments in the future.

Samantha Johnson, MA, CCLS

Stanford Children's Hospital / Palo Alto, CA

Sam works as a Certified Child Life Specialist III at Stanford Children's Hospital. She began her career in radiology before transitioning to the pediatric intensive care unit for seven years, where she supported trauma, neurology, psychiatry, palliative care, pulmonary, and general pediatrics service lines. Sam was the primary Specialist for all cystic fibrosis patients. She currently works in the oncology and hematology outpatient unit. She is also the handler of a facility dog, Margene.

Sam completed her Bachelor of Arts in English and Anthropology at the University of Colorado at Boulder and earned her Master of Arts in Early Childhood Education with an Emphasis in Child Life in Hospitals from Mills College. Sam resides in Northern California with her husband and two sons. In her free time, she enjoys visiting the ocean, painting, and reading.

Nam Soo Joo, PhD *

Stanford University / Palo Alto, CA

Dr. Joo has been working in the CF research field, with a special emphasis on a potential role played by airway submucosal glands in the process of CF lung disease since he joined the cystic fibrosis research laboratory, directed by Dr. Jeffrey Wine, at Stanford in 1999. Since then, he has contributed to the development of important methods in collaboration with colleagues at Stanford: an in situ optical gland secretion assay for measuring fluid secretion rates and compositions from individual airway submucosal glands; a bioluminescence assay for bacterial killing with saliva samples; proteomic assay of gland mucus from CF and control human airways; airway smooth muscle contraction assay and mucociliary clearance (MCC) assay for measuring a mucociliary clearance velocity using ex vivo WT and CF animal tracheae. With these methodologies, he discovered defects in submucosal gland secretory functions in CF airways from human and CF animal models. Recently, he discovered synergistic MCC in ex vivo animal tracheae by combining β adrenergic and low dose cholinergic agonists (synergy agonists, SA), including CF ferrets, without inducing airway narrowing. SA produced synergistic MCC

in vivo sheep model of CF airway. Outcomes of preclinical clinical safety and tolerability trials in healthy and CF subjects indicated that SA is safe to use in humans. To better understand the underlying mechanisms of synergistic enhancement of MCC, experiments are underway: using ex vivo MCC assays-ENaC involvement; changes in airway surface liquid (ASL) height - synchrotron method; intracellular [Ca2+]i levels -ratiometric and GCaMP methods; viscoelasticity - dynamic light scattering microrheology assay; and in situ gland secretion assay with WT and CF animals. The outcome of these studies would provide important information necessary to develop SA as a potential therapeutic for patients with airway muco-obstructive disorders, including CF.

Nicholas Kelly, MS, RD, LD

Cleveland, OH

Passionate, energetic, knowledgeable, and compassionate, are a few words to describe Nicholas Kelly's approach to life. His story began at three months old when his mother diagnosed him with cystic fibrosis. Growing up, Nick strove for normalcy, valuing himself as a person, meant to stand out.

Nick thrived despite his disease, obtaining a Bachelor's and Master's degree from Bowling Green State University, and becoming a dietitian. In addition, he is an author, decorated speaker, and advocate. Nick's life has focused on utilizing his abilities and knowledge to influence those around him while acting as a positive representation of the educational, artistic, and CF community.

For more information, visit www.NicholasKellyRD.com

Luanne McKinnon, PhD

Albuquerque, NM

Luanne McKinnon (b. 1955) is approaching 70 years with cystic fibrosis. Over the course of her life, she has been attended at various CF centers, including Dallas, where she was diagnosed, New York City, Charlottesville, Edinburgh, Scotland, Albuquerque, and Palo Alto. She served as the first co-chair of the Stanford Patient and Family Advisory Committee with Elyse Elconin-Goldberg. In 2011, Luanne underwent a successful bi-lateral lung transplant at Stanford under the care of doctors David Weill and Gundeep Dhillon.

Having thrived through myriad breakthrough medications and modes of self-care, she has had a rich career in the visual arts for over forty-five years. Ms. McKinnon holds a PhD in the History of Art from the University of Virginia; and an MFA in Painting from TCU in Fort Worth. She owned McKinnon Modern, a private art dealership in New York City specializing in European and American masterworks. Returning to academe, Luanne served as the Bruce A. Beal Distinguished Director at the Cornell Fine Arts Museum at Rollins College and Director of the University of New Mexico Art Museum. Her expertise has yielded several publications in the field, and she has mounted over thirty-five exhibitions. She was a Fellow at the Getty Research Institute.

Luanne McKinnon is married to the Emmy-award winning filmmaker, Daniel Reeves. They share one daughter, Adele Reeves de Melo of London.

Speaker Profiles

Robin Modlin, MA

Livermore, CA

Robin Modlin has been a part of CFRI for over 40 years. She has served on the board and as an editor for the newsletter. Ten years ago, Robin initiated CFRI's Embrace, an annual retreat for mothers of children and adults with CF. Embarking on the task to write a book about her CF journey as a mother and the remarkable story of her daughter, Anna, Robin says, "writing the book was a way for me to tell our story in an artful way. Our family has had a beautiful, challenging and amazing life and Anna is a living miracle."

As an emerging author, Robin writes with an introspective point of view influenced by her interest in psychology, spirituality, and creativity. As an expressive artist, Robin creates individual and community mosaics and facilitates SoulCollage® workshops. She is looking forward to sharing her story with the CF community she cares so much about.

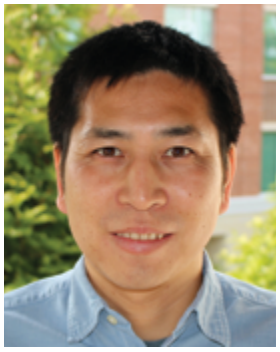
For more information, visit www.robinmodlin.com

Richard B. Moss, MD

Stanford University / Palo Alto, CA

Dr. Moss, Professor Emeritus of Pediatrics at Stanford University, is former chief of the pediatric pulmonary and allergy divisions, and former allergy-immunology and pulmonary fellowship training programs director at Lucile Packard Children's Hospital Stanford. He was educated and trained at Columbia (BA), SUNY Downstate (MD), Children's Memorial Hospital of Northwestern University (pediatric residency) and Stanford (allergy-immunology and pulmonology fellowships). He was Director of the Stanford Cystic Fibrosis Center from 1991 to 2009 and a principal investigator for the Cystic Fibrosis Foundation's Therapeutics Development Network, where he also served as inaugural Chair of the Protocol Review Committee. He is a member of Stanford's Child Health Research Institute and has served on Stanford's Pediatric Mentoring Program for trainees and junior faculty, the Executive Committee of Spectrum Child Health (Stanford's NIH-funded clinical research program) and the Stanford IRB.

Dr. Moss has reviewed and consulted for the NIH, CFF, national and international foundations, and many peer-review bioscience journals and biopharmaceutical companies. He has published over 250 research papers and is a frequent speaker at national and international medical conferences. His research interests have included pathogenesis, outcome measures, and treatment of chronic airway diseases of childhood such as asthma, CF and chronic lung disease of infancy, with an emphasis on mechanisms of pulmonary immunity, inflammation and allergy. Recent work has focused on allergic fungal lung disease and clinical testing of novel CF tests and treatments. He joined CFRI's Board of Directors in 2015.



Kenichi Okuda, MD, PhD *

University of North Carolina / Chapel Hill, NC

Dr. Okuda obtained his MD degree from Yamagata University in Japan and completed residency training in internal medicine, followed by a fellowship in respiratory medicine. He then pursued postdoctoral training in Dr. Richard Boucher's laboratory at University of North Carolina (UNC) at Chapel Hill, where he successfully characterized regional expression patterns of major airway secretory mucins, MUC5AC/MUC5B, and CFTR/ionocytes in normal and CF human airways. This work culminated in the completion of his PhD degree in medicine from The University of Tokyo in Japan. During his research, Dr.

Speaker Profiles

Okuda developed microdissection techniques to selectively isolate small airway tissues, enabling the generation of small airway epithelial cell/explant cultures for studying small airway-specific biology and physiology. These techniques have been integral to numerous collaborative projects nationwide, including the Human BioMolecular Atlas Program (HuBMAP). In 2021, Dr. Okuda was promoted to an Assistant Professor in the Department of Medicine, Division of Pulmonary Diseases and Critical Care Medicine at UNC at Chapel Hill. His long-term career goal is to serve as a professional investigator, dedicated to comprehensively understanding the mucociliary clearance system in the lungs and contributing to improved prognoses for all patients with muco-obstructive lung diseases, including cystic fibrosis.

Matthew Porteus, MD, PhD *

Stanford University / Palo Alto, CA

Matthew Porteus MD, PhD is the Sutardja Chuk Professor of Definitive and Curative Medicine, Director of the Center for Definitive and Curative Medicine, and a Professor in the Department of Pediatrics, Institute of Stem Cell Biology and Regenerative Medicine and Maternal-Child Health Research Institute at Stanford. His primary research focus is on developing genome editing as an approach to cure disease, particularly those of the blood (such as sickle cell disease) but also of other organ systems as well.

Dr. Porteus received his undergraduate degree at Harvard in History and Science where his honors thesis studied the recombinant DNA controversy of the 1970s. He then completed his MD and PhD training at Stanford, clinical training in Pediatric Hematology/Oncology at Boston Children's Hospital, and post-doctoral research training with Noble Laureate David Baltimore at CalTech. He works as an attending physician on the Pediatric Hematopoietic Stem Cell Transplant service at Lucile Packard Children's Hospital where he cares for children undergoing bone marrow transplantation for both malignant and non-malignant diseases. His goal is to combine his research and clinical interests to develop innovative curative therapies. He served on the 2017 National Academy Study Committee of Human Genome Editing and currently serves on the Scientific Advisory Board for WADA on Cell and Gene Doping and the NIH NexTRAC advisory committee evaluating the emergence of new technologies.

Amy Ryan, PhD

University of Iowa / Iowa City, IA

Amy Ryan, PhD, is an Associate Professor of Anatomy and Cell Biology and Associate Director of the Center for Gene Therapy at the University of Iowa. Her research program focuses on understanding the complex mechanisms that contribute to the pathogenesis of lung disease with a particular focus on cystic fibrosis and ciliopathies. The long-term goal of her research program is to fully understand specification of specialized airway epithelial cell types from stem and progenitor cells in the human lung in the context of lung injury, regeneration and disease pathogenesis. To date, she has published 56 peer-reviewed original research papers, 45 invited reviews/editorials/chapters and edited a book focusing on Lung Stem Cells. Dr. Ryan has extensive experience in both pulmonary and stem cell biology with specific training in lung physiology and pathology including the vasculature and airways, electrophysiology, gene editing, stem cells and regenerative medicine. Her major contributions to the field include developing differentiation protocols, mimicking embryonic lung development, to specify cells comprising the proximal airway epithelium. She is now using these induced pluripotent stem cell (iPSC)-based models, in conjunction with primary airway epithelial stem cells, to understand mechanisms of injury and repair of the human airway epithelium, including, but not limited to, progenitor cell specification and differentiation, epithelial barrier function, injury and repair and mucociliary clearance.

Speaker Profiles

Recently, her lab has expanded their focus to understand the impact of the cellular niche, including roles of inflammation and inflammatory cells, in airway regeneration. Inflammation is a core pathogenic process in many lung diseases, including cystic fibrosis, and is likely to influence cellular behaviors during airway regeneration. Dr. Ryan recently showed that crosstalk between neutrophils and the airway epithelium results in significantly enhanced inflammatory response to infection with SARS-CoV-2 when compared to either cell type studied in isolation. The development of more complex, multi-cellular and tissue-level models is likely to provide more mechanistic insight into tissue specific changes in regeneration in response to disease or infection. In collaboration with Dr. Parekh at the University of Iowa, and as part of the Cystic Fibrosis Foundation's Consortium on Stem Cells, Dr. Ryan is further developing pre-clinical large animal models for evaluating cellular based therapeutics for airway disease. Established projects in this area involve generating lung epithelium and mesenchyme from iPSC using both 2-D and 3-D differentiation models mimicking embryogenesis as well as isolation, culture and comparisons to iPSC-derived and primary basal cells. In the context of restoration of functional mucociliary clearance, Dr. Ryan's research is focused on characterizing novel mechanisms controlling multiciliogenesis using both human iPSC-based and primary lung epithelial cell-based models to manipulate specific genes and pathways during specification of these cells. Tissue level responses are being evaluated in intact ex vivo tissues as well as human airway-on-chip models developed in her laboratory.

Zachary Sellers, MD, PhD *

Stanford University / Palo Alto, CA

Dr. Sellers is a pediatric physician-scientist and research and clinical development consultant. As a pediatric gastroenterologist and ion channel physiologist, Dr. Sellers' work in academia and pharma over the last 20 years has focused on improving the lives of individuals with complex and rare diseases through providing cutting-edge clinical care and advancing research and drug development. Dr. Sellers previously led a basic and translational research laboratory at Stanford, focused on epithelial ion transport and acid-base regulation using a variety of human and animal models. He is a firm believer in the exponential impact of team science and is adept at working in multi-disciplinary and cross-functional teams. He seeks out strategic partnerships and opportunities that can leverage his expertise and leadership to advance innovative therapies for areas of high unmet need and to support the development of the next generation of physician-scientists.

Dr. Sellers received his BS (Animal Physiology and Neuroscience) and BA (Japanese Studies) from the University of California. San Diego, his MD and PhD (Molecular and Integrative Physiology) from the University of Illinois at Urbana-Champaign, and was trained in Pediatrics and Pediatric Gastroenterology, Hepatology, and Nutrition at Stanford, where he was previously a faculty member and attending physician. He held multiple leadership positions at Stanford, including Associate Chief of Research in Gastroenterology, Director of the Stanford Children's Pancreas Program, Lead Gastroenterologist for the CF Program, Director of the CFTR Phenotyping and Theratyping Program, and Physician-Scientist Advisor for the Pediatrics Residency Program.

Speaker Profiles

Jennifer Taylor-Cousar, MD, MSCS

National Jewish Health / Denver, CO

Dr. Taylor-Cousar is a tenured professor of adult and pediatric pulmonary medicine at National Jewish Health (NJH), where she serves as the Medical Director of Clinical Research Services, President of the Medical Staff, and is co-director of the Adult Cystic Fibrosis (CF) Program and Director of the CF Therapeutics Development Network (TDN) center. She received her undergraduate degree in human biology from Stanford University, and completed her Doctorate in Medicine, combined residency in internal medicine and pediatrics, and her combined fellowship in adult and pediatric pulmonary medicine at Duke University. She obtained her Master of Clinical Science from the University of Colorado.

Dr. Taylor-Cousar's expertise is clinical trial design and conduct; she has been national/global primary investigator on multiple CF TDN pharmaceutical trials. Her investigator-initiated research focuses on the development and evaluation of novel therapies for the treatment of CF, and the long-term impacts of these therapeutics on health outcomes in people with CF. She has published more than 130 manuscripts as well as a book and multiple book chapters. She is an elected member of the American Society for Clinical Investigation (ASCI). Additionally, she serves on a number of national scientific advisory committees for Emily's Entourage, the Cystic Fibrosis Foundation, American Thoracic Society and the National Institutes of Health. She is an Associate Editor for the Journal of Cystic Fibrosis and a member of the International Advisory Board for the Lancet Respiratory Medicine. She is also a staunch advocate for racial justice both in academic medicine and for people with CF.

Natalie E. West, MD, MHS

Johns Hopkins University / Baltimore, MD

Natalie E. West, MD MHS, Assistant Professor of Medicine of Pulmonary and Critical Care Medicine at Johns Hopkins University, serves as the Associate Program Director of the Adult Cystic Fibrosis (CF) Program. Dr. West's expertise is in clinical trial design and conduct; she has been a co-investigator in multiple clinical trials in CF pulmonary exacerbations as a national investigator in the Standardized Treatment of Pulmonary Exacerbations (STOP) program. She has been integral in the design and conduct of three large trials through the STOP program. Additionally, Dr. West is one of 5 national investigators who founded the Sexual Health, Reproduction, and Gender Research (SHARING) Working Group, who has identified gaps in knowledge around health issues that impact women with CF and developed the infrastructure needed to conduct multiple clinical studies. Dr. West has published expert opinions and guidelines, spoken at national and international conferences, and has conducted numerous clinical trials in both areas, to understand the long-term impacts on health outcomes in people with CF.

Katrine Whiteson, PhD *

University of California Irvine / Irvine, CA

Dr. Katrine Whiteson is an Associate Professor at the University of California, Irvine, and co-Director of the UCI Microbiome Center, with over 15 years of experience in microbiome research. She earned her BA in Biochemistry from UC Berkeley and her PhD from the University of Chicago. Dr. Whiteson's research focuses on understanding the human microbiome's role in health and disease, particularly in cystic fibrosis (CF). She explores innovative approaches to combatting CF pathogens, including the use of bacteriophages, antibiotics, and small molecule adjuvants. Her lab hunts for phage that can attack CF-

Speakers Profiles

pathogens, especially *Stenotrophomonas*, in feces, sewage, sputum and other well-loved samples – the students are brave! Her interdisciplinary work aims to develop tailored therapies for CF patients, with the ultimate goal of improving treatment outcomes and quality of life.

For more information, visit Dr. Whiteson's lab website: http://faculty.sites.uci.edu/whitesonlab/

Kate Yablonsky, LCSW
Stanford Health Care / Palo Alto, CA

Kate Yablonsky joined the Stanford Adult CF Team in 2018. She is also the social worker for Stanford's Interstitial Lung Disease program. Prior to that, she spent more than the first decade of her career in social work at Lucile Packard Children's Hospital working with the pediatric oncology and stem cell transplant teams. She did her undergraduate degree at New York University and got her master's in social work from UC Berkeley with a concentration in health.

In addition to her day-to-day work as a CF social worker, Kate facilitates support groups for CFRI for adults with CF, pediatric CF caregivers, and adult CF caregivers and spouses. She has also facilitated groups for CFRI conferences and Miles for CF/ CF Lifestyle Foundation (now BreatheStrong CF). The first person with CF she ever met was the younger brother of a close childhood friend; he was her first window into this special community and is still a huge reason she is so inspired to come to work every day. He is well into adulthood and doing great! Kate is married to a 5th grade teacher and has a daughter and a son. She is originally from Pittsburgh, PA.

Panelist Profiles

Rachel Alder / *Salt Lake City, UT*

Rae Alder is a fierce advocate and has been since her early childhood years. Subsequently, Rae crafted a life's work centered in the art of advocacy and authenticity. The majority of her career has been spent in diversity victim advocacy working with survivors of sexual assault, domestic violence, and human trafficking. Rae has also been a public speaker since her early high school years, from panels to trainings to Keynotes, speaking on ethics regarding foster care and transracial adoption. She is a National Champion (2022) in After Dinner Speaking for her University, and her academic pursuits include neuroscience and social work with an emphasis on trauma and neuroplasticity.

Rae recently overcame racial bias, health disparity, and rapid health deterioration to finally receive the correct diagnosis of cystic fibrosis in January 2023 at the age of 26. Subsequently, Rae has shifted her career focus to patient advocacy particularly in regard to bioethics, health equity, and for all marginalized folks facing cystic fibrosis. Rae firmly believes in the power and importance of sharing our own authentic journeys. These are her greatest passions.

Rae serves on the Board of Directors of the Utah Pride Center as well as the Bonnell Foundation Board of Directors. She serves on the Cystic Fibrosis Foundation Adult Advisory Council and CFRI's CF Adult Advisory Committee and Diversity and Inclusion Advisory Committee.

Panelist Profiles

Jaelyn Cooper, MHS / *Little Rock, AR*

Jaelyn is a 28-year-old native of Little Rock, Arkansas, diagnosed with cystic fibrosis (CF) at 18 months old. Jaelyn has both a Bachelor's degree and Master's degree in Health Services Administration. In her spare time, she loves reading, roller skating, playing instruments, traveling, and attending concerts.

Jaelyn's journey with CF has taught her the importance of speaking up and advocating for herself. She hopes that every person with CF receives the utmost respect, quality care, and understanding. This is why she wants to actively participate in the mission to ultimately find a cure.

Elyse Elconin Goldberg, MA / *Los Gatos, CA*

Elyse was diagnosed with cystic fibrosis at the age of three. She attributes her longevity to a lifelong commitment to exercising, being exceedingly compliant with her medical regime and excellent care from her medical team. Ten years ago, she had a double lung transplant.

Elyse graduated from UC Davis with a degree in Urban Planning (1980) and received a Master's degree from the University of Southern California in Urban and Regional Studies (1981). After relocating to the Bay Area, she began a career in Marketing Communications working for several start-up companies. She left the high-tech industry in 1993 to start a family. She is married and has two grown children. Recently, she became a grandmother!

Elyse currently serves as Chair of the Lung and Heart/Lung Transplant Patient and Family Advisory Council at Stanford. She has served on the CFRI Board of Directors and is currently a member of the CF Adult Advisory Committee and the CFRI Retreat Committee. Elyse and her husband Craig have been married for 39 years. They enjoy travel, the arts, hiking adventures, pickleball and spending time with our expanding family. Elyse is an avid pet enthusiast and loves time spent with her Maltipoo pup Teddy.

Thomas Horal / *San Jose, CA*

Thomas, diagnosed with cystic fibrosis at the age of five, has shown remarkable resilience throughout his life. He has dedicated much of his time to volunteering to support domestic violence survivors and the elderly. Though facing many health challenges as a young adult, he has since experienced meaningful improvements with Trikafta. Last year, Thomas encountered another challenge with a colon cancer diagnosis at the age of 31. Currently in remission, Thomas is continuing his journey of strength and dedication while actively participating in CFRI Advocacy events.

Colleen Lewis / *Philadelphia, PA*

Colleen is an adult with cystic fibrosis and a breast cancer survivor. She owns and operates a thriving dog boarding business in Philadelphia, where her love for animals shines through in the personalized care she provides. Colleen teaches yoga classes for the CF community, when her mind and body allow, through CFRI and CF Yogi.

Outside of work, Colleen enjoys hiking with her dogs and tending to her rose garden, blending her love for nature with her usually active lifestyle. She's currently in a phase of recovery and rest, while her body adjusts to ongoing cancer treatments. Find her on Instagram **@calminphilly**

Panelist Profiles

Alicia Maciel, MBA / *Brea, CA*

Alicia Maciel is a leading member of the Cystic Fibrosis Research Institute's Diversity and Inclusion Committee, and the Embrace Mother's Retreat Planning Committee. She edits and translates CFRI written resources and videos to Spanish. Alicia served on the Family Advisory Council at Children's Hospital of Orange County. She lives in Southern California with her husband and two young adult sons, one of whom was diagnosed with cystic fibrosis at age six.

Alicia is a certified Leadership Coach and Mindfulness Mentor with an MBA from Harvard Business School, a multicultural perspective, and over 28 years of management experience across industries. Alicia specializes in supporting others to build resilience and cultivate wellbeing. She serves as part-time faculty at Cal State University Fullerton teaching Organizational Behavior and Team Leadership courses. People who know Alicia describe her as kind, intuitive, genuine, and resourceful. Her hobbies include being out in nature and creating mixed media art.

Christine Nash, MBA / *Lafayette, CA*

Christine is a strategic advisor and Board member in the biopharma industry focusing on helping companies bring new medications to market for the treatment of rare diseases. Her most recent full-time role was Chief Commercial Officer for Hyperion Therapeutics, a company focused on the treatment of urea cycle disorders. She earned a BA in Public Policy and an MBA, both from Stanford University.

In 2004 Christine, her parents, and her brother and sister-in-law co-founded the Elizabeth Nash Foundation in honor of her CF-affected sister Liz who died at age 32. The foundation seeks to improve the lives of people affected by CF through its scholarship program and CF research grants.

Abhijit Tirumala / *Saratoga, CA*

Abhijit "Abhi" Tirumala is a 22-year-old CF patient with a nonsense mutation living in the Bay Area. He graduated from Santa Clara University last spring with a B.S. in Neuroscience, and just finished his year-long service term with National Health Corps San Francisco. He is interested in and passionate about biomedical science and wants to become a physician in the future. He was diagnosed at the age of three, and he has an older brother who also has cystic fibrosis. His brother was diagnosed at the age of five. From his personal experience, he has not met many South Asian individuals that also have cystic fibrosis. Therefore, he hopes to be able to help increase South Asian representation and awareness within the cystic fibrosis community, and he wants to advocate for the importance of diversity in clinical trials. He is appreciative and thankful to CFRI to be able to participate in this panel, so as to share his experiences of living with cystic fibrosis.

Panel Moderators

Jean Hanley, MD

Dr. Jeanie Hanley is a physician living with cystic fibrosis. She has been married for 38 years and is mother to three adult children. Although she had CF symptoms since childhood, she was diagnosed in her early 30's only after genetic testing became available. Three of her nine siblings also have CF.

Advocating for patients and families has been a passion of hers. As an allergist at USC in Los Angeles, she spearheaded the implementation of the national Breathmobile® Program, a comprehensive, mobile allergy and asthma program treating medically-underserved children at their schools. Dr. Hanley also founded a nonprofit patient advocacy organization called Planning Health. She currently works in the greater Los Angeles community as an allergist in private practice.

She has been dedicated to volunteering for CF organizations such as CF Roundtable, where she was President for 5 years and with CFRI, where she serves on the Board of Directors, devotes time to committees involved in promoting CF Research and Advocacy and moderates a virtual support group for adults with a late CF diagnosis.

Kimberly Morse, MSW, LCSW

Kimberly Morse, MSW, LCSW, is a CF social worker at Children's Hospital Los Angeles (CHLA). She earned her Master of Social Work degree at University of Washington with a concentration in Health/Mental Health in 2001. Kimberly has spent her twenty year (and counting) social work career working with a variety of ages and medical conditions, in medical settings across the U.S, including CHLA since 2011.

Kimberly has worked in cystic fibrosis (CF) over the past thirteen years at CHLA and is inspired by the resiliency of many patients and families. Kimberly has received CF Foundation grant funding to support mental health and has participated in nationwide CF quality improvement networks. Kimberly has presented at national CF conferences and CHLA workshops on topics related to mental health, DEI and health care, transition, ethical dilemmas, and managing challenging behaviors. Kimberly has been a CFF SW Mentor for five apprentices, is a current clinical supervisor and former field instructor for MSW interns, to both support and learn from, the next generation of social workers.

Support/Discussion Group Facilitators

Olin Dodson, MA

Olin Dodson, MA, is a father and retired Licensed Professional Clinical Counselor in Santa Fe, New Mexico. He facilitates CFRI's monthly Grief to Growth support group on Zoom. Olin first connected with CFRI in 1990 after he learned his Costa Rican daughter, Melissa, had been diagnosed with cystic fibrosis. After the end of their seven years together, he wrote a memoir recounting the story of their shared life and went on to teach a series of workshops, "Cross Cultural Approaches to Grief" with Native American Social Worker Jennifer Nanez (Acoma). Over time, he's learned the immeasurable value of personal stories shared between members of the diverse and resilient CF community.

Olin's writing has appeared in TheRavensPerch, Trickster Literary Journal and the Santa Fe New Mexican. When he is not writing, hiking, or schmoozing with his adopted children, Sasha and Gisell, he tutors middle school students in poetry and creative writing.

Sonya Haggett, LCSW

Sonya is a licensed clinical social worker from the San Francisco Bay Area living with cystic fibrosis, who is eight years post-double lung transplant. She has served CFRI over the years as group facilitator for the Summer Retreat and Educational Conference and as a member of the Summer Retreat Committee. She facilitates CFRI's monthly support groups for adults with CF post-transplant and the newly launching support group for those ineligible/unable to use CFTR modulator therapies.

Deborah Menet, LCSW

Debbie Menet, LCSW, is a Licensed Clinical Social Worker in the Cystic Fibrosis Center at Stanford Medicine Children's Hospital. She has over 20 years of experience working with children, adolescents and families in both school-based and medical settings. Besides her work at Stanford, Debbie also has a private psychotherapy and therapeutic yoga practice, providing support to people impacted by medical conditions, healthcare workers, and people moving through a life transition. Besides her love for her work, Debbie also enjoys being active in Northern California's beautiful environment.

You can find more information about Debbie and her work at www.DebMenet.com

Kimberly Morse, MSW, LCSW

Kimberly Morse, MSW, LCSW, from Children's Hospital Los Angeles (CHLA), earned her Master of Social Work degree at University of Washington with a concentration in Health/Mental Health in 2001. Kimberly has spent her twenty year (and counting) social work career, working with a variety of ages and medical conditions, in medical settings across the U.S, including, CHLA since 2011. Kimberly has worked in cystic fibrosis (CF) over the past thirteen years at CHLA and is inspired by the resiliency of many patients and families. Kimberly has received CF Foundation grant funding to support mental health and has participated in nationwide CF quality improvement networks. Kimberly has presented at national CF conferences and CHLA workshops on

Support/Discussion Group Facilitators

topics related to mental health, DEI and health care, transition, ethical dilemmas, and managing challenging behaviors. Kimberly has been a CFF SW Mentor for five apprentices, is a current clinical supervisor and former field instructor for MSW interns, to both support and learn from, the next generation of social workers.

Teresa Priestley, MSW

Teresa is one of the pediatric social workers at Stanford Children's cystic fibrosis clinics. She has been a parent-educator for almost 20 years. She has extensive experience working with parents and children who have experienced trauma and/or separation. One of the main objectives of her work is to empower parents to help their children heal from pain and trauma that they have experienced. She really enjoys assisting families in problem-solving.

Alanah Rosenbloom, LCSW

Alanah Rosenbloom is an adult living with cystic fibrosis. She has grown alongside CFRI at each stage of her life; from appearing in a fundraising campaign as a child in the 1980's, to attending CFRI's National CF Education Conference as a teenager, and loving the Adult Retreat into her 20's and 30's. She earned her BA in Communication at the University of California Davis and Master's Degree in Social Work from San José State University. She currently works in hospice care. Alanah is an only child with several "inherited" nieces and nephews she adores. She enjoys cooking, reads mostly non-fiction, and likes listening to podcasts.

Ann Steiner, PhD

Dr. Steiner is a nationally recognized psychotherapist, trainer, consul-tant, and author with a private practice in Lafayette. Since giving a keynote address for CFRI in 2000, she has returned every year to lead support group facilitators and to facilitate groups. A dynamic and powerful speaker for over 25 years, she has presented throughout the U.S. and internationally for medical and mental health organizations, consumer groups and other illness-related organizations. She is passionate about the healing power of group work, is a Fellow of the American Group Psychotherapy Association, helped found and consults for the Group Therapy Training Program for The Psychotherapy Institute in Berkeley. Her book, *Help Your Group Thrive: Workbook and Planning Guide* for leaders of work groups, writer's groups, book clubs, community and networking groups, Routledge Press, was recently translated and published in Chinese by Green Pasture, Hong Kong. Drawing on her personal and professional experience with chronic conditions, Dr. Steiner produced a CD, *The Rollercoaster of Chronic Illness: How to Add Joy to the Ride*, which is also the title of her soon to be published self-help book. **(visit www.drsteiner.com)**

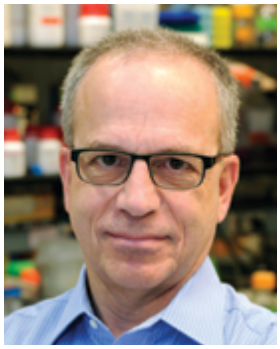
Support/Discussion Group Facilitators

Kate Yablonsky, LCSW

Kate joined the Stanford Adult CF Team in 2018. She is also the social worker for Stanford's Interstitial Lung Disease program. Prior to that, she spent the first decade+ of her career in social work at Lucile Packard Children's Hospital working with the pediatric oncology & stem cell transplant teams. She did her undergraduate degree at New York University and got her Masters in Social Work from UC Berkeley with a concentration in health.

In addition to her day-to-day work as a CF social worker, Kate facilitates support groups for CFRI for adults with CF, pediatric CF caregivers, and adult CF caregivers and spouses. She has also facilitated groups for CFRI conferences and Miles for CF/ CF Lifestyle Foundation. The first person with CF she ever met was the younger brother of a close childhood friend; he was her first window into this special community and is still a huge reason she is so inspired to come to work every day. He is well into adulthood and doing great! Kate is married to a 5th grade teacher and has a daughter and a son. She is originally from Pittsburgh, PA.

2024 CFRI Awards and Awardees



The 2024 Paul M. Quinton Cystic Fibrosis Legacy Award

John LiPuma, MD** / **University of Michigan

John LiPuma is Research Professor of Pediatrics and Communicable Diseases, Division Director, Pediatric Infectious Diseases Research Program at the University of Michigan. His research laboratory focuses on bacterial infections of the airways in persons with cystic fibrosis. His laboratory has developed novel methods of bacterial genotyping to study the molecular epidemiology of CF related respiratory tract pathogens, as well as numerous genetic-based methods for bacterial species identification. His interest in bacterial taxonomy has contributed to the description of dozens of novel bacterial species. His work focusing on bacterial pathogenesis has utilized cell culture and animal models of infection. Recent research employs culture-independent microbial community profiling, including the use of next generation DNA sequencing, to understand the airway microbiota in CF. His laboratory serves as a national reference laboratory for the Cystic Fibrosis Foundation, and maintains an extensive culture collection that includes approximately 40,000 bacterial strains. With the support of CFRI, Dr. LiPuma has studied the epidemiology of Burkholderia Cepacia. Dr. LiPuma is recognized as one of the most impactful researchers in CF microbiome studies with a research record second to none in the world of CF microbiology.

The 2024 CFRI Partners in Living Award in Memory of Anabel and Isabel Stenzel

Laura Mentch, EdM

Laura was diagnosed with cystic fibrosis at the age of 50, after years of being told that allergies and asthma were the cause of her repeated lung and sinus infections. After her diagnosis, Laura immediately became an engaged member of the national CF community. CFRI's retreat and conference were among her earliest connections, and she immediately became an active participant in CFRI events. Laura has lived on both the East and West Coasts, but now hails from Bozeman, Montana. Prior to her diagnosis, Laura received her graduate degree, married, and raised three children while working as a health educator – with a strong emphasis in sexual and reproductive health. Laura has served as a key interface between CFRI and the CF Reproductive and Sexual Health Collaborative (better known as CFReSHC). Laura serves on the Board of Directors of the United States Association of CF Adults (USACFA) CF Roundtable, as well as the Cody Dieruf Foundation in Montana. In 2023, she joined CFRI Board Member Dr. Jeanie Hanley in serving as a co-facilitator of CFRI's support group for Adults with a Late CF Diagnosis. Prior to her passing, Isabel Stenzel Byrnes nominated Laura for this award, and CFRI's Board of Directors celebrate Laura and her many contributions to the cystic fibrosis community.

The 2024 David Stuckert Memorial Volunteer of the Year Award

Alicia Maciel, MBA

Alicia Maciel's impact upon CFRI is profound. The mother of two sons, one of whom has cystic fibrosis, Alicia has been a highly involved volunteer for many years. Alicia has volunteered as an editor of CFRI's Spanish language newsletter for nearly nine years, assisting with content and ensuring accurate translations. She serves on CFRI's Diversity and Inclusion Advisory Committee where she has played a pivotal role in CFRI's reach to the Hispanic/Latinx community. She has participated in our diversity films on CF in the Hispanic/Latinx community, recording podcasts in Spanish to help Spanish-speaking members of our community access CFRI's resources. She helps to coordinate diversity workshops for our community members and has facilitated a webinar for CF social workers on mindfulness and DEI work. Alicia

2024 CFRI Awards and Awardees

also serves on CFRI's Embrace Mothers Retreat Committee, where she plays a pivotal role in assisting with event planning and content. Alicia is generous with her time and expertise. She is always ready to support her fellow CF community members and is an outstanding CFRI volunteer.

The 2024 CFRI CF Professional of the Year Award

Kate Yablonsky, LCSW

Kate Yablonsky, LCSW joined the Stanford Adult CF Team as the CF Social Worker in 2018. She started as a social worker at Lucile Packard Children's Hospital over ten years ago in the Pediatric Stem Cell Transplant Program, before moving to the CF center. Kate sees her patients beyond their diagnosis with cystic fibrosis. Originally from Pittsburgh, Pennsylvania, Kate earned her undergraduate degree at New York University her Master's in Social Work from UC Berkeley. She is known for her caring kindness and respect for her patients, as well as her patience and humor. Kate has been involved with CFRI for many years. She is a co-facilitator of CFRI's CF Adult Support Group and the two CF Caregivers Support Groups. She has presented at CFRI's conference as well as in CFRI webinars. Kate is always generous with her time. The first person with CF she met was the younger brother of a close childhood friend; he was her first window into the CF community. In an interview, Kate directed her comments to adults living with CF, saying, "Trust yourselves… You have grown up with an incredible burden that has forced you to experience and contemplate things at a very young age that some people never have the courage to face. The result is a community of highly intuitive, thoughtful, resilient people. You understand yourselves, your bodies, and the world in a way that others do not. Trust that." This response demonstrates Kate's respect for her patients and why she is the recipient of this award.

Remembering Ann Robinson and Danielle Mandella

Ann Robinson, MA, first volunteered with CFRI in 1979, two years after the birth of her second child, Carl, who was diagnosed with cystic fibrosis. At that time, CFRI was only four years old and led by a small, very dedicated group of parents. Ann and her husband, William (aka Rob), became vital members of this group, bringing their passion and talents to help advance CFRI's mission. Ann and Rob both served many spots on CFRI's Board of Directors, and Ann became the organization's first executive director. Through the years, Ann mentored countless parents of children with CF. She played a critical role in CFRI's organizational development, and in the lives of countless members of our CF community as she empowered families to receive optimal care. Her crown jewel was the National CF Education Conference. After her retirement from CFRI, Ann remained a dedicated CFRI volunteer. Ann passed away in December 2023. Her profound impact upon our CFRI community endures.

Danielle Mandella, who lived with cystic fibrosis, passed away in November, 2023 at the age of 37. Danielle was a very rare living donor lung transplant recipient, having received a lobe from each of her parents in 2003. Those lungs sustained her for over 20 years, but chronic rejection and other transplant-related complications claimed her life. Danielle volunteered with CFRI for many years on CFRI's Retreat Committee and CF Adult Advisory Committee prior to joining CFRI's staff as the Social Media Associate. In that role she expanded CFRI's reach around the globe while launching the now annual Dance Like A Fool event to support CFRI's virtual wellness classes. Danielle was a committed advocate for those with cystic fibrosis and other disabilities. A talented improv artist, Danielle possessed a sharp wit and dry sense of humor. Danielle lived with grace, intent and focus until her last breath.

Presentation Abstracts

*** denotes CFRI-funded researchers**

* Role of IRBIT in Ion Transport Trafficking in Healthy and Cystic Fibrosis Intestine

Friday, July 26, 9:00 am

Zachary Sellers, MD, PhD
Stanford University, Palo Alto, CA

Proximal intestinal pH is acidic in cystic fibrosis and may contribute to impaired digestion and absorption of nutrients. We found that the heat-stable enterotoxin of E. coli and its therapeutic analog linaclotide can raise duodenal pH by stimulating duodenal bicarbonate secretion. Linaclotide achieves this by increasing apical membrane expression and activity of the down-regulated in adenoma (DRA) chloride/bicarbonate exchanger. I will discuss a series of experiments in which we explored the mechanism(s) whereby linaclotide achieves this, including identifying linaclotide's effect on the cellular localization of inositol 1,4,5-triphosphate (IP3) receptor binding protein released with IP3 (IRBIT) and its potential role in ion channel trafficking in healthy and cystic fibrosis duodenum.

* Improving CF Airway Mucociliary Clearance: Toward Transition from Animals to Humans

Friday, July 26, 9:55 am

Nam Soo Joo, PhD
Stanford University, Palo Alto, CA

Airway mucociliary clearance (MCC) plays a critical role in host innate defense, and defective mucus clearance contributes to many obstructive airway diseases, including cystic fibrosis (CF), primary ciliary dyskinesia, chronic obstructive pulmonary disease, asthma, chronic rhinosinusitis, and idiopathic pulmonary fibrosis. The present study focuses on MCC in CF. Impaired MCC is a primary defect of CF airway innate defense that causes bacterial infection, inflammation, and mucus obstruction. We recently discovered in ex vivo experiments that the combination of two classes of agonists (synergy agonists, ß-adrenergic + low dose cholinergic) produces near-maximal mucociliary clearance velocity in tracheae from ferrets and newborn piglets without inducing airway constriction. Importantly, in the tracheae from transgenic CF ferrets, we also found synergistic clearance. The concept, however, seems counterintuitive as a response to ß-adrenergic (a cAMP agonist) is missing or defective in people with CF and cholinergic (a Ca2+ agonist) is known to cause airway constriction. For these reasons, the therapeutic possibilities of the combined agonists might have escaped notice. In the conference, in vivo and ex vivo MCC data by the synergy agonists, a mechanistic aspect of synergistic MCC, and its potential for a clinical development to improve lung function in people with CF will be presented and discussed.

* Pf Bacteriophage is Associated with Antibiotic Tolerance and Poor Outcomes in Patients with Cystic Fibrosis

Friday, July 26, 11:05 am

Paul Bollyky, MD, PhD
Stanford University, Palo Alto, CA

Pf phage, a bacteriophage associated with *Pseudomonas aeruginosa* (Pa) infection, is abundant in the airways of many individuals with cystic fibrosis (CF). We have identified roles for Pf phage in biofilm formation and antibiotic sequestration. Further, the presence of Pf phage in sputum from individuals with CF has been linked to more severe exacerbations and increased antibiotic resistance in cross-sectional cohorts. Here, we have investigated how Pf phages and sputum biopolymers impede antibiotic diffusion using human sputum. Our analysis suggests that Pf phages in sputum reduce the diffusion of charged antibiotics due to a greater binding constant associated with organized liquid crystalline structures formed between Pf phage. Consistent with this, in a longitudinal study involving individuals with CF, we find that Pf is associated with accelerated loss of lung function, suggesting a potential role for Pf phage in the pathogenesis of CF lung disease. Pf phage may serve as a prognostic biomarker and potential therapeutic target in CF.

Presentation Abstracts

*A Hybrid Gene Correction Strategy for Cystic Fibrosis

Friday, July 26, 12:45 pm

Anais Amaya, PhD; Matthew Porteus, MD, PhD
Stanford University, Palo Alto, CA

Recent advancements in small molecule correctors and modulators have significantly improved treatment for most cystic fibrosis (CF) patients. However, a percentage of CF patients do not benefit from these treatments. Moreover, modulator therapy requires daily administration. A proposed therapeutic approach that could result in durable correction would be to repair the genetic defect at the DNA level using genome editing tools like CRISPR/Cas9. Our group has successfully optimized the process of editing upper airway basal cells using ex vivo editing techniques. However, successfully engrafting these cells back into the airways remains a challenge. A potential solution is adjusting the delivery technique to enable direct mutation correction within the lung tissue itself. In this study, we describe our efforts to develop viral and non-viral editing reagents to correct the most common CF-causing mutation in precision-cut human lung slices (PCLS). There are two components of the gene correction system that we need to deliver to lung tissue: the CRISPR/Cas9 nuclease with the sgRNA and the donor template to be used by the cell to correct the genetic defect. We evaluated various delivery strategies including lipid nanoparticles (LNPs), virus-like particles (VLPs), lentiviral vectors (LVs), adeno-associated viral vectors (AAVs) and cell-penetrating peptides (CPPs) for delivery to lung slices. For delivery of the donor vector, we used two different strategies to identify AAV serotypes with high tropism towards lung cells and particularly basal cells within PCLS. In the first approach, we directly compared various serotypes, both natural, such as AAV2, AAV5, and AAV6, and engineered ones specifically designed for lung tissue, such as AAV2.5T. In a second approach, we used a barcoded library for high-throughput screening of 81 known AAV variants. We have identified vectors that can be efficiently used to deliver therapeutic reagents to human lung. These reagents could later be used towards ex vivo gene editing of lungs or lobes of lung and enable clinical translation.

*Triple Whammy: Targeting Recalcitrant CF Pathogens with Phages, Antibiotics, and Small Molecule Adjuvants

Friday, July 26, 1:50 pm

Katrine Whiteson, PhD
University of California, Irvine, CA

Interest in phage therapy has skyrocketed due to the continued emergence of antibiotic-resistant bacteria and a relatively limited pipeline of truly novel antibiotics. Recent successes in using phages to treat otherwise untreatable infections have generated significant interest, particularly within the cystic fibrosis (CF) community. Despite their promise, phages alone often fail to eradicate recalcitrant infections, as bacteria can evolve resistance and other pathogens may exploit the resulting ecological niches. To address this, we are developing synergistic cocktails that combine phages, antibiotics, and small-molecule adjuvants. We are testing clinical isolates of recalcitrant CF pathogens in polymicrobial communities and under conditions that mimic CF airway environments.

Our lab has been actively hunting for phages in Southern California wastewater to target clinical isolates of antibiotic-resistant CF pathogens, including *Stenotrophomonas maltophilia* and *Pseudomonas aeruginosa*. We have developed a high-throughput 96-well plate liquid synergy assay to test how CF-pathogens respond to combinations of phages and small molecules. This assay is being applied to evaluate synergy between phages, antibiotics, and a large variety of small molecules with putative bacteriocidal and phage enhancing activity. So far, we have tested multiple alcohols, butanedione/diol, multiple essential oils, and many other molecules. Perhaps our biggest success has been with short and medium-chain fatty acids (SCFAs and MCFAs), which are thought to increase cell membrane permeability. Overcoming challenges with solubility and pH control, we have obtained promising results with saturated straight-chain fatty acids up to 10 carbons and are now exploring longer-chain and branched fatty acids.

We find that all tested fatty acids limit growth of *S. maltophilia*, however SCFA inhibition is primarily dependent upon pH, and does not improve phage performance. This stands in contrast to MCFAs, which show pH-independent growth inhibition and act synergistic with phages. Using octanoic acid as a proxy, the bactericidal and phage enhancement activity of MCFAs seems to be universal, that is it

works with multiple phages, and both gram negative bacteria (such as *S. maltophilia*) and gram-positive bacteria (such as *Enterococcus*). We are currently expanding our work with MCFAs to include conditions more relevant to the CF airway (such as variations in oxygen and the use of Artificial Sputum Media), and extending to other CF pathogens, including *Staphylococcus aureus*, and *Pseudomonas aeruginosa*.

*CFTR High Expresser Cells Are pH-Sensing Neuropods

Friday, July 26, 2:45 pm

Nadia Ameen, MBBS
Yale School of Medicine, New Haven, CT

CFTR High Expresser cells (CHEs) are a rare (1-2%) subpopulation of enterocytes in rat and human small intestine that express high levels of CFTR and bestrophin 4 (BEST4). ScRNA-seq in human intestine and preliminary data in rat jejunum identified CHE-specific genes encoding transcription factors, proton and anion channels, acid sensors, motors, neuropod cell function. Neuropods are sensory epithelial cells that engage in fast neurotransmission onto neurons and the enteric nervous system (ENS). CHE-specific proteins were visualized in rat jejunum cryosections by immunofluorescence (IF) of BEST4, a Cl-/HCO3- channel, synaptotagmin 3 (SYT3), beta tubulin 3 (TUBB3), and choline acetyltransferase (CHAT), all relevant to ENS function, beta tubulin 2b (TUBB2B), involved in vesicle transport, S100A6, a calcium-binding protein, Meis homeobox 1 (MEIS1), a CHE-specific transcription factor, and guanylyl cyclase C (GCC), a receptor that regulates acid-stimulated HCO3- secretion. IF of CFTR, MEIS1, GCC, and the proton channel otopetrin 2 (OTOP2) were performed in cryosections after in vivo luminal exposure to normal or low pH. Fluorescence in situ hybridization (FISH) of CFTR and GUCA2B, that generates the endogenous ligand to GCC, was conducted on normal and DF508 rat jejunum sections to elucidate mRNA levels and localization in CHEs. CHEs were identified with BEST4+ basal processes close to neurons expressing SYT3 and TUBB3. CHEs were selectively innervated by axons expressing CHAT and enriched for cytosolic TUBB2B and S100A6 in the apical domain. ScRNA-seq revealed CHE-specific enrichment of the acid sensing receptor GPR4. CHEs robustly traaicked CFTR from subapical endosomes to the brush border and OTOP2 was highly upregulated in the apical domain of CHEs under low luminal pH. Functional studies in rat organoids using pHrodoTM Red supported OTOP2-dependent proton transport in CHEs. CFTR mRNA levels, enriched in CHE nuclei and intestinal crypts, were reduced in DF508 rat intestine while GUCA2B, ubiquitous in the cytoplasm of CHEs, was unchanged. CFTR protein was barely detected in the cytoplasm and absent from the apical domain of DF508 CHEs. CHE abundance was increased 3-fold in DF508 rat jejunum, suggesting a compensatory response in the CF intestine. Interestingly, Best4+/MEIS1+ cells were detected in CFKO rat jejunum, indicating that CFTR was not critical to the fate of the cells. These findings reveal CHEs to be neuropod cells that sense and respond to their environment, especially to luminal pH, supporting a critical role for CHEs in CF intestinal disease pathogenesis.

*Molecular Regulatory Pathways Associated with the Distinct Identity of Large and Small Airway Epithelia in Humans

Friday, July 26, 3:45 pm

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

Background: Airway mucociliary clearance (MCC) is a crucial innate defense mechanism for lung health. Dysfunction of MCC is a major pathogenic factor in cystic fibrosis (CF). Small airway regions are particularly susceptible to mucus plugging in CF. However, the specific contributions of regionally distinct epithelial cells to integrating intraregional MCC remain poorly understood. This study aimed to elucidate the molecular mechanisms determining the distinct identity of large and small airway epithelial cells by characterizing region- and cell-type specific chromatin accessibility profiles in primary human large and small airway epithelial (LAE and SAE, respectively) cells.

Methods: Fully differentiated in vitro primary human LAE and SAE cells obtained from previously healthy donor lungs were dissociated and utilized for snRNA/ATAC-seq assays. Specific transcription factors (TFs) such as NKX2-1 and FOXA2 were inactivated by CRISPR/Cas9 technology in SAE and LAE cultures to determine their role in maintaining small airway epithelial identity.

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Results: A total of 18,031 cells (LAE 43.4%, SAE 56.6%) passed quality control. snRNA-seq identified an SAE-enriched subset of secretory cell clusters expressing high levels of SFTPB, SCGB3A2 and RNASE1, in addition to previously reported major and rare airway epithelial cell types present in both LAE and SAE cultures. snATAC-seq identified region- and cell-type specific accessible chromatin regions and associated TF motifs. Notably, NKX2-1 motifs were uniquely enriched in the small airway secretory cells. Subsequently, we tested whether NKX2-1 regulates small airway secretory cell identity, utilizing CRISPR/Cas9 technology. Immunohistochemistry demonstrated a reduction in the frequency of small airway secretory cells and an increase in MUC5AC+ goblet cells in NKX2-1 KO SAE, but not in NKX2-1 KO LAE, cells. Bulk mRNA-seq revealed increased levels of goblet cell marker expression (MUC5AC, FOXA3, ITLN1) and decreased levels of small airway secretory cell marker expression in NKX2-1 KO SAE cells, consistent with immunohistochemistry quantitation. Importantly, higher mucus concentration was observed in NKX2-1 KO SAE compared to control cultures, suggesting disrupted SAE mucus homeostasis due to NKX2-1 inactivation.

Conclusions: The integration of snRNA/ATAC-seq from in vitro primary human LAE and SAE cells identified region- and cell-type specific open chromatin regions and associated TFs. Small airway cellular identity is regulated in part by NKX2-1, and loss of NKX2-1 function disrupted homeostatic secretory cell phenotype in SAE cells. These findings provide novel insights into the regulatory mechanisms determining the regional identity of human airway epithelia that may be associated with maintaining airway homeostasis in health and dysfunction in CF.

* CFTR mRNA Delivery to Cystic Fibrosis Airways

Friday, July 26, 4:35 pm

Beate Illek, PhD
University of California, San Diego, CA

Background: The first description of the chloride impermeability in the reabsorbing sweat glands of cystic fibrosis (CF) patients (Quinton, 1986) has paved the way for CFTR-directed therapeutics to treat the underlying chloride and HCO3- channel defect in CF. Although CF is a monogenetic disease, 719 CFTR mutations are known to cause CF (cftr2.org). The CFTR gene spans 188,702 base pairs with twenty-seven exons (and twenty-six introns), and its mature transcript is 6,129 nucleotides long with an open reading frame of 4,440 nucleotides which provides the code for the 1,480 amino acids of the CFTR protein. Theratyping is a relatively new concept to group CF genotypes more precisely based on their responsiveness to CFTR-directed therapeutics. As of today, 178 mutations are eligible for CFTR modulator therapy and an additional 376 may become eligible based on a threshold for restoring 10% of normal CFTR function in preclinical assays. It becomes clear that further studies are needed to address whether function can be restored above 10% in carriers with one or two of the remaining CF-causing mutations; if not, mRNA-based CFTR therapy could provide an alternative and universal approach. Currently, phase 1 clinical trials using lipid nanoparticles for the delivery of mRNA encoding CFTR are ongoing for VX-552, ARCT-032, and RCT2100, but was halted for MRT5005 due to clinical challenges.

Results: For preclinical studies, we developed 2D airway epithelial cell models to assess the responsiveness to CFTR-directed therapeutics with the Ussing assay, which is considered the gold standard. CF cells were obtained from pediatric or adult CF patients by nasal brushings and an initial harvest of as few as 20,000 cells was sufficient to expand up to fifty million cells and generate air-liquid interface cultures. Short-circuit current measurements discriminated CFTR function between healthy control subjects (wild type, WT) and CF patients with intermediate (F508del/R117H-7T: 56% WT) and severe mutations (F508del/F508del: 12% WT). CFTR activity for R334W/406-1G>A was 24% WT, F508del/c.850dupA was 12% WT, and CFTRdele2,3(21 kb)/CFTRdele2,3(21 kb) was 9% WT. The homozygous CFTRdele2,3(21 kb) unexpectedly responded to VX-661 treatment (20% WT). Genotypes that were clearly below the 10% threshold included W1282X/W1282X (1% WT), G542X/1248+1G>A (2% WT), G85E/1717-1G>A/ (0.7% WT) and V520F/1717-1G>A (3% WT) suggesting CFTR mRNA delivery as an alternative approach. Delivery of mRNA by various LNP formulations showed robust GFP expression in sub-confluent CF bronchial epithelial cells but not confluent CF monolayers suggesting that the apical cell membrane can be rate-limiting for effective mRNA delivery.

Conclusions: 1) The homozygous CFTRdele2,3(21 kb) genotype may be considered for label expansion based on its in vitro modulator responsiveness above 10%. 2) Confluent CF cell monolayers may present useful test models to evaluate and improve delivery of CFTR mRNA therapeutics.

The Power of Passion

Friday, July 26, 6:15 pm

Nicholas Kelly, MS, RD, LD
Cleveland, OH

Do you know what it's like to be told you have a chronic disease, what it takes to fight despite obstacles, and thrive in the face of adversity? That is Nick's life.

Nick's story starts at three months old, when he was diagnosed with cystic fibrosis. Although interesting, this is not what made his story unique. Nick was actually diagnosed by his mother, a fact we will get into later. Despite his diagnosis Nick went on to thrive, becoming a dietitian, speaker, author, artist, advocate and much more.

Nick's advocacy is something he holds dear, as he loves to share knowledge and passion. He believes in the importance of being informed. As his favorite quote says, "A candle doesn't lose anything lighting another candle." Nick hopes to be the light that dispels some of the darkness surrounding CF. He is dedicated to shining a light on research, addressing inadequacies, advocating for the community, and driving the mission to make CF stand for "Cure Found."

Book Reading: *Love, Courage and Miracles*

Friday, July 26, 8:15 pm

Robin Modlin, MA
Livermore, CA

Embark on a heartwarming journey through this tale of love, courage, and miracles. Join Robin as she confronts her daughter Anna's life-limiting prognosis, navigates the challenges of cystic fibrosis, and witnesses Anna's miracle resurrection through a double lung transplant. Together, they defy the odds and are fueled by hope, gratitude and a quest for meaning and healing.

Follow them as they meet a Tibetan lama who opens their world with ancient healing rituals, have an audience with the Dalai Lama, and in one of her darkest hours, Anna experiences a healing blessing from a magical blue budgie. With her newfound lungs, Anna triumphs, realizing unexpected dreams and invites exciting adventures into their lives.

Love, Courage, and Miracles is more than a story. It is a testament to the unbreakable bond between a mother and daughter entwined with the challenges of a devastating disease. Their journey is a powerful narrative of not just survival but living life to the fullest, showcasing the transformative power of love, acceptance, and hope.

* Phage Therapy for CF-Associated Infections *

Saturday, July 27, 9:00 am

Benjamin Chan, PhD
Yale University, New Haven, CT

Bacteriophage (phage) therapy, the therapeutic administration of the bacteria killing viruses, has been successfully deployed to treat multi- and pan-drug resistant pulmonary infections. Cystic fibrosis (CF) is a disease complicated by recurrent, often antibiotic-resistant infections, and as a result, is at the forefront of the development of phage therapy. One aspect of phage therapy not typically explored is the consequence of phage-resistance both on bacterial populations and secondary effects on the human body. For example, genetic changes impacting the production of proinflammatory metabolites. Given the inevitable evolution of phage-resistance, studies focusing on these changes and their potential clinical impact are of critical importance. We examined the impact of three phage that utilize virulence factors as receptor binding sites on strains of *Pseudomonas aeruginosa* collected from sputum of individuals treated with these phage. These phage utilize either the O antigen, Type IV pilus, or core

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LPS as their receptor binding sites and, as a result, potentially drive a genetic tradeoff between phage resistance and virulence. We quantified the impact of these phage on the expression of these virulence factors after exposure to phage in culture as well as on bacterial density in sputum following phage therapy. We also measured the impact of phage therapy on the pulmonary microbiome in these cases and further quantified the impact of phage therapy on forced expiratory volume (FEV1). We found a significant decrease in production of targeted virulence factors in single-phage treated samples, a significant reduction in bacterial density in sputum following phage therapy, and significant increase in FEV1. These results, while preliminary, are encouraging and further work will be critical to determine the duration of these effects.

Modeling Epithelial Immune Cell Interactions in Cystic Fibrosis

Saturday, July 27, 10:05 am

Amy Ryan, PhD
University of Iowa, Iowa City, IA

Mucociliary clearance is a key mechanical defense mechanism of human airways, and clearance failure is linked to major respiratory diseases, including cystic fibrosis. Despite Highly Effective Modulator Therapy (HEMT) benefits, persistent lung inflammation and compromised pathogen clearance in people with CF (pwCF) suggests inadequate targeting of lung innate immunity or potential drug tolerance. These issues highlight a need to further understand the interplay between immune cells and airway epithelial cells during injury, repair, and regeneration.

Our research focuses on developing models to elucidate these dynamics. We have recently generated lung-on-chip models incorporating CF primary bronchial epithelial cells and elucidated the structural parameters of airway epithelia that predict clearance function in both ex vivo and in vitro tissues. From these we developed physics-based models to translate measurable parameters to quantitatively benchmark the human-relevancy of mucociliary clearance in experimental models, and to characterize distinct disease states.

Furthermore, we have engineered ΔF508 mutant THP-1 cells, using CRISPR/Cas9 technology, to investigate the role of CFTR (Cystic Fibrosis Transmembrane Conductance Regulator) in innate immune functions. We compared isogenic wild-type, ΔF508 mutant, and CFTR knock-out THP-1 cells, differentiated into macrophage-like cells, and tested their responses with CFTR modulators (ivacaftor, elexacaftor, tezacaftor). Our findings indicate dose-dependent effects of these modulators on inflammatory responses, TNFα release, phagocytosis, bacterial killing, cell migration, and expression of pro-inflammatory markers in both ΔF508 and wild-type macrophages. Notably, lower concentrations of modulators attenuate inflammation, while higher doses, especially in combination, may exacerbate immune dysfunction.

These insights into how HEMTs impact macrophage function highlight potential mechanisms underlying immune complications observed in CF patients despite treatment. Future investigations incorporatingpatient-derived macrophages could reveal additional therapeutic targets to mitigate these challenges. Moving forward, our integrated approach combining physics-based models, gene-edited immune cells, and lung-on-chip technologies promises to deepen our understanding of inflammation's role in airway stem cell function and functional mucociliary clearance. This multidisciplinary framework holds promise for advancing personalized cellular therapies for CF and other lung diseases.

Aging in the New Age of Cystic Fibrosis

Saturday, July 27, 11:15 am

Richard Moss, MD
Stanford University, Palo Alto, CA

In the 15 years since the clinical trials of Kalydeco® (ivacaftor) in G551D-carrying people with CF (~4% of the total CF population) showed a potential transformative early benefit, confirmed and vastly expanded by the effects of the triple drug Trikafta® (elexacaftor/ tezacaftor/ivacaftor, ETI) on 85-90% of people with CF in the Global North, the entire worldwide CF community has come to realize that we have entered a new age, where the potential of a full lifespan is now predicted for those diagnosed, eligible and on CFTR modulator treatment from an early age. What the rosy predictions may neglect is

the realization that aging with, instead of prematurely dying from, CF presents its own set of formidable new challenges, for lifelong "highly effective" CFTR modulator therapy [HEMT] with ETI is not a cure.

Aging on top of defective, not fully corrected CFTR function and established disease impacts on many organs despite HEMT, recent studies confirm, is associated with a panoply of many CF- (and transplant-) related complications and early vulnerability to an expanding array of age-associated diseases such as diabetes, a variety of cancers, cardiovascular disease, obesity, osteoporosis, and other health challenges. The huge issue of the ineligible minority with HEMT-unresponsive CFTR mutations remains a new mountain to climb. In addition, there is increasing recognition of the further problems of HEMT intolerance and heterogeneity of response, CF underdiagnosis in much of the world and among many ethnicities (partly due to genotypic heterogeneity and partly to many socioeconomic factors), lack of access to HEMT in many countries and exorbitant lifelong cost. In this overview talk I will endeavor to address many of these aspects of our new age of CF, and present CFRI's new programmatic efforts to raise expertise of caregivers for aging CF patients as well as providing accurate information and empowerment to all people with CF everywhere.

Culture Shift: CF Lung Infections in the Modulator Era

Saturday, July 27, 1:15 pm

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

CFTR modulator therapy has dramatically changed the pace, course, and overall nature of CF lung disease for many, but not yet all, people with CF. Even before these transformative treatments were introduced, the "ecology" of CF airway infections - the types and prevalences of different bacteria in the respiratory samples from people with CF - had changed considerably over the years as treatments, social practices, and hospital policies changed. At the same time, our understanding of how microbes infect people with CF in the first place, and how those microbes adapt and change during infections, has grown enormously, aided by new technologies, concepts, and insights from a variety of fields. We are only now updating these models of CF lung infection pathogenesis in the context of CFTR modulator treatments, with some studies suggesting the need for large-scale revisions of how we diagnose, treat, and think about these infections, guided both by ongoing research and what our patients are telling and showing us.

In this presentation, I will review the "cast of characters" – the microbes that are most frequently detected and other less common but equally important ones – involved in CF airway infections. We'll see how these infections have changed over the years, as treatments and policies have changed. I'll talk about the benefits and risks of current treatments, especially antibiotics, and the meaning of antibiotic resistance, and how these concepts might change in the context of CFTR modulators. In addition, I'll discuss the implications of the ongoing decreasing rates of expectoration among many people with CF, a trend that preceded the introduction of highly-effective CFTR therapy but that has greatly accelerated since. In particular, I will review what this trend means for diagnosing CF respiratory infections, and work on the horizon to improve detection of infections in this new era.

Sexual and Reproductive Health in CF

Saturday, July 27, 2:20 pm

Natalie E. West, MD, MPH
Johns Hopkins University, Baltimore, MD

With improved therapies, people with cystic fibrosis (CF) are living longer and healthier lives. People with CF have an increasing number of questions regarding their sexual and reproductive health. This talk will summarize important issues during puberty, adulthood, and menopause that specifically affect people with CF.

A wide range of sexual and reproductive health topics including puberty, transgender and gender nonbinary identities, contraception, cyclical hemoptysis, fertility, contraception, and parenthood will be addressed.

More people with CF are expressing the desire to become pregnant, as people with CF are living longer lives. In the last 4 years, the pregnancy rate of women with CF has tripled in the United States. The

impact of highly effective modulators has improved the health of many people with CF, which is allowing them to consider all reproductive options. Care during pregnancy, management of CF medications during pregnancy, and outcomes with the use of highly effective modulator therapy during pregnancy and lactation will be discussed, as there are retrospective studies available, as well as a large ongoing prospective pregnancy clinical trial. 95-97% of men with CF have congenital bilateral absence of the vas deferens, which leads to infertility in men with CF. Assisted reproductive technology is available which can be used to assist in having biological children.

Other options for family building include fostering, adoption, and surrogacy. Gaps in knowledge, current evidence, and management strategies to optimize care in people with CF will be discussed. The impact of the approval and increased use of highly effective modulator therapy on sexual and reproductive health care needs and outcomes remains to be seen. However, due to the positive impact on health and longevity, it is expected that people with CF will increasingly face concerns and decisions. Optimizing sexual and reproductive health care as the face of CF changes is imperative to meet these emerging needs throughout the lifespan.

Panel: Coping with Cancer and CF

Saturday, July 27, 3:30 pm

Elyse Elconin Goldberg, MA; Thomas Horal; Colleen Lewis; Christine Nash
Los Gatos, CA; San Jose, CA; Philadelphia, PA; Lafayette, CA
Moderated by Jean Hanley, MD
Manhattan Beach, CA

As adults with cystic fibrosis are living longer, additional health complications are emerging. Some of these are related to having CF; others are made more complex by a CF diagnosis. There is increased awareness of the higher risk of certain cancers for adults with CF, most notably gastrointestinal cancers. There is a higher risk of pancreatic cancer; the risk of colon cancer for those with cystic fibrosis is five to ten times higher than that of the general population. Breast cancer rates are higher for women with CF, with evidence of the role of sex hormones, particularly estrogen, impacting the pathophysiology of CF.

For individuals with CF who have received a double lung transplant, the risk grows significantly; colon cancer risks are over 25 times higher than the general population due to the immunosuppressants required to ward off organ rejection. With the immune system suppressed, cancers are more likely to develop and spread, most significantly GI, esophageal and skin cancers. In this session, panel participants share their personal experiences with diverse cancer diagnoses. A sibling will describe her brother's battle with pancreatic cancer, while three adults with CF will describe their individual journeys with breast cancer, skin cancer and colon cancer. Awareness, detection and early intervention are key. Panelists will share insights and perspectives on detection and treatment, as well as the mental health implications of this dual diagnosis.

Panel: Advocacy, Access and Health Equity in Cystic Fibrosis

Sunday, July 28, 9:15 am

Rachel Alder; Jaelyn Cooper, MHA; Alicia Maciel, MBA; Abhijit Tirumala
Salt Lake City, UT; Little Rock, AR; Brea, CA; Saratoga, CA
Moderated by Kimberly Morse, MSW, LCSW
Children's Hospital Los Angeles, Los Angeles, CA

Despite the fact that cystic fibrosis occurs in every race and ethnicity, there remains a misperception – both in the general public, and among medical care providers - that the disease only impacts people of European descent. As a result, many people of color with CF are misdiagnosed for years, and are thereby unable to benefit from CF therapies and care. People of color are more likely to have rare mutations that are missed by states' newborn screening panels, exacerbating the likelihood of a late diagnosis. And these rare mutations are far less likely to be responsive to CFTR modulator therapies, thereby leaving fewer therapeutic options. Current CF therapies were largely approved through clinical trials with very few

people of color participating. For CF patients and families for whom English is not a first language, it may be challenging to access resources and support. During this dynamic panel discussion, we will hear from members of our CF community who will share their personal experiences and insights navigating bias to develop productive partnerships with care teams and fellow community members so as to improve medical care and quality of life. All panelists provide examples of optimal care and interactions, while stressing the importance of self-advocacy and community engagement.

Exploring Nucleic Acid Based Approaches to Treat People with CF

Sunday, July 28, 10:15 am

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

Cystic fibrosis (CF) was first described as a clinical entity by Dr. Dorothy Andersen in 1938. At that time, people with CF unfortunately did not survive past early childhood. In subsequent years, the establishment of comprehensive care and treatments directed at the signs and symptoms of CF improved the quantity and quality of lives for people with CF. In 1989, the CF transmembrane conductance regulator (CFTR) gene was discovered; it was thought that the development of gene therapy to cure CF would be imminent. However, in early gene therapy clinical trials, ineffective vector (transport mechanism) transduction (transfer of the corrected gene) into lower airway cells along with immune-mediated side effects temporarily prevented advancement of this approach to treating CF.

While deficits in early gene therapy technology precluded its initial development for CF, focus shifted to addressing the downstream protein dysfunction caused by variants in the CFTR gene. Since 2012, four CFTR protein modulators have been approved. In populations comprised primarily by people of European descent, >90% of people are variant eligible for CFTR modulators. However, side effects and access preclude CFTR modulator use by some variant-eligible people with CF. Critically, there are also people with CF whose variants make them unable to benefit from CFTR modulators. To achieve effective therapies for all people with CF, nucleic acid based therapies are being developed.

The goal of nucleic acid based therapies (NABT) is to deliver the correct instructions to the cell for making a functional CFTR protein. Examples of nucleic acid based therapy include gene editing (making specific changes to the gene), gene therapy (replacing the entire gene), mRNA [messenger ribonucleic acid] therapy (using the corrected blueprint for the protein) and antisense oligo nucleotide (ASO) therapy (using a very small amount of matching RNA to correct the blueprint). The various approaches are each associated with advantages and challenges including potential variant-agnostic treatment (e.g. therapies that are effective regardless of CFTR variant), differences in delivery requirements for each potential therapy, potential immune reactions to therapeutic delivery of the therapies, and possible barriers for re-dosing. Nonetheless, gene therapy, mRNA and ASO treatments are currently in clinical trials for pwCF.

In this session, we will review lessons learned from historical NABT efforts, pre-clinical data supporting renewed clinical investigative efforts, and ongoing clinical trial designs and updates.

Strategies to Address Medical Trauma

Sunday, July 28, 11:20 am

Samantha Johnson, MA, CCLS; Kate Yablonsky, LCSW
Stanford Children's Medicine, Palo Alto, CA; Stanford Health Care, Palo Alto

Over the course of a lifetime with chronic illness and interaction with the medical system, people with cystic fibrosis and their families are at high risk of experiencing medical trauma. This is an under-discussed but very real form of trauma that can have a significant impact on quality of life. In this session, we will review signs and symptoms of medical trauma and discuss strategies for people with CF and their loved ones across the lifespan to effectively process and integrate these experiences.

Presentation Abstracts

Living Proof: Nearly Seventy Years with Cystic Fibrosis *Sunday, July 28, 12:20 pm*

Luanne McKinnon, PhD

Albuquerque, NM

Luanne McKinnon, PhD (b. 1955) has cystic fibrosis and is a thirteen-year survivor of a successful bi-lateral lung transplant. Her presentation, "Living Proof: Nearly 70 Years with Cystic Fibrosis" is a colorful recounting of her life with CF, from a diagnosis in 1969 through the arc of CF care that, like a travel log, leads us from Texas to Europe to New York City, Scotland and France, then New Mexico to Palo Alto. She will speak about her uncanny good fortune to live at a time, in the long history of CF, in which research and healthcare have mitigated so much suffering. Ms. McKinnon's presentation weaves a brief historical account of the discovery of CF in 1938 into the broader, compassionate scope of living under the "double rainbow" of hope and doing the work required to be here now.

"Living Proof" will be presented in two parts. Part 1 is a personal testimony. Part 2 is an imaginative piece about the life of her mother's sister who died in 1935 from symptoms echoing CF. Inspired by the late Isa Stenzel Byrnes, who during one of her last creative writing workshops posed the question, "How do we honor those we have lost?" Ms. McKinnon will share an excerpt from her memoir-in-progress entitled, Pneuma, Latin for soul and breath. The CFRI audience will be the first to hear this.

Luanne McKinnon received an MFA in Painting from TCU in Fort Worth, Texas; a PhD in Art History from the University of Virginia, Charlottesville; and is an authority on Pablo Picasso and particularly, his masterwork, Guernica, 1937. She has been a two-time museum director; has curated over thirty-five exhibitions; and is a noted writer on contemporary art. Her exhibition, "Eva Hesse Spectres 2010" received accolades in the New York Times and over sixty other publications and premiered in 2010 at UCLA, six months before her lung transplant. She is currently maintaining a steady schedule as an independent writer and editor; and last year profited from workshops with the Pulitzer Prize writers, Forrest Gander and Gregory Pardlo. She was the first co-director, with Elyse Elconin-Goldberg of Stanford's Lung and Heart-Lung Patient and Family Advisory Committee. Luanne McKinnon lives with her husband, an Emmy-award winning filmmaker, Daniel Reeves in Albuquerque and Sigoules, France. They share a daughter, Adele Reeves de Melo of London.

Special Thank You

CFRI Professional and Volunteer of the Year Awards Panel

Francine Bion
Zoe Davies, NP
Colleen Dunn, MS, RT, CCRD
Oscar Flamenco, CPA
Marina Gonzales
Carole Nakamura, RN
Dennis Nielson, MD, PhD
Anna Payne
Deepika Polineni, MD, MPH
Yelizaveta Sher, MD, FACLP

CFRI Conference Committee

Francine Bion
Sabine Brants, MA
Mary Convento
Barbara Curry
Leeya Kannankunni
Jennifer Keller, MD
Rohini McKee
Jane Mitchell, MSW
Stacie Reveles
Siri Vaeth, MSW

Support & Discussion Groups

CFRI's virtual Support and Discussion Groups offer an opportunity to gather with CF community peers to share experiences and information that are unique to those touched by cystic fibrosis. This year, we will offer separate in-person and virtual support groups:

- *Adults with CF*
- *Parents/Caregivers of Children with CF*
- *Parents/Spouses/Partners/Siblings of Adults with CF*
- *Adults with CF post-transplant*

Please read the guidelines below to understand what you can expect from our support and discussion groups and what we expect from group participants.

- CFRI Support and Discussion Groups are designed to bring people together to facilitate support, camaraderie, and information sharing. We do not offer individual or group therapy in the support groups, and this is not an opportunity for counseling, diagnosis, or treatment of specific disorders.
- Please be prepared to commit to a minimum of 45 minutes with your selected group.
- Confidentiality is important to all attendees. To ensure confidentiality, you are asked to not reveal participants' names or their personal issues outside of the group.
- There will be a facilitator for each group whose biographical information is listed in the conference program. Facilitators are required by law to report incidences of child, elder or spousal abuse.
- Respect the members of your group, including their situations, emotions and perspectives. Limit making suggestions to others unless they ask for ideas and advice.
- Please give quieter members an opportunity to share.
- It is okay to listen and remain silent. Simply say, "pass," if the group is sharing and it is your turn.
- If you want to discuss an uncomfortable experience with the medical system, leave out names.
- In many groups, attendees like to share and trade medical information. The final word about any medical treatment should come from your/your family member's own physician.

Awards Celebration Special Guests

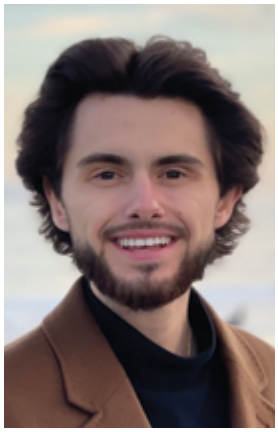
Paul Quinton, PhD

Dr. Paul Quinton's seminal cystic fibrosis research advanced understanding of the disease and has had a pivotal impact on the field. Dr. Quinton, who has CF himself, discovered that the basic defect in the CF sweat duct was due to anion impermeability and not defective anion exchange. Quinton's laboratory at the University of California San Diego investigated the mechanisms of normal and pathophysiological functions in affected epithelia, including the control and role of CFTR in ion secretion and absorption processes, and the interaction of electrolytes with mucins. Dr. Quinton has served as an inspiring mentor to others in the field. He has been an active member of the CFRI community for decades, and currently serves on CFRI's Research Advisory Committee and CF Adult Advisory Committee.

Gunnar Esiason

Gunnar Esiason is a cystic fibrosis and rare disease patient leader, who is passionate about early-stage drug development, patient empowerment, antimicrobial resistance, and health policy. Gunnar has a BA from Boston College, a Master of Business Administration from the Tuck School of Business at Dartmouth and a Master of Public Health from the Dartmouth Institute for Health Policy and Clinical Practice. He has consulted on clinical trial development, a real-world evidence population health study, and a CF-specific mental health screening tool. His health policy opinions have been featured in leading news sources, Including the Wall Street Journal, USA Today, The Hill, and STAT News.

Dance Party DJ



Dylan Dunn

As the sibling of Tess, who lives with cystic fibrosis, and as the son of Siri, the director of CFRI, Dylan has a deep understanding of the challenges faced by those living with the disease, and also of the amazing and inspiring CF community. A graduate of Sonoma State University with a BA in Psychology, Dylan is a realtor with Coldwell Banker in Santa Cruz County, California, focused on residential sales. Music has always been his passion, from creating songs to serving as DJ at weddings and celebrations. Nothing makes him happier than seeing people on the dance floor, and he always ensures people hear the songs that inspire them to move. He is honored to DJ CFRI's annual dance party.

Help Us Pursue Our Mission

Partners in Living ~ Research for Life

DONATE TO THE JESSICA FREDRICK MEMORIAL CF RESEARCH CHALLENGE FUND — Thanks to our generous Jessica Fredrick Memorial CF Research Challenge Circle donors, any gift made to the Jessica Fredrick CF Research Challenge Fund will be matched 100%. All contributions will be restricted to CF research awards granted through the New Horizon and Elizabeth Nash Memorial Fellowship programs.

TRIBUTES IN HONOR OF, AND IN MEMORY OF — Any gift to CFRI can be made in honor or in memory of a loved one. Your loved one's name will appear in our newsletter, CFRI Community, and if requested, an acknowledgement will be sent to the person you designate.

MOTHERS' DAY CELEBRATION — Our Mothers' Day Celebration supports our research, education and advocacy programs. We provide inspiring cards to send to friends, colleagues and family members, or participate via our virtual campaign. It is fast, easy and very meaningful!

DONATE YOUR BIRTHDAY (OR OTHER SPECIAL EVENT) TO CFRI ON FACEBOOK — Setting up a birthday event on Facebook is free and easy, and 100% of the donations go directly to CFRI. Simply go to Facebook.com/cfri.org, scroll to the "Fundraisers" section and click on "Create." Facebook birthdays have become an important source of support for CFRI's services.

GIVING GIFTS OF STOCK TO CFRI — Giving a gift of appreciated stock to CFRI is easy and rewarding. You will not pay capital gains tax on stock that has appreciated over the years, and will receive an income tax charitable deduction for the fair market value of the stock on the date of the gift. If you wish to donate stock certificates to CFRI, contact us for instructions on how to complete the transaction.

ATTEND A CFRI FUNDRAISING EVENT — Whether you want to golf, wine taste, or bid on exclusive auction items, we have something special for you! Upcoming events include:

- The 40th Annual Golf Tournament Benefitting CFRI at the beautiful Cinnabar Hills Golf Club in San Jose on Monday, August 12, 2024.
- CFRI's Gala, "A Breath of Fresh Air," will be held Sunday, October 6, 2024, in-person at the breathtaking Nestldown Estate (Los Gatos, CA). Sponsorships are available!

VEHICLE DONATIONS — If you have a car, boat, recreational vehicle or motorcycle that you no longer need, please consider donating it to CFRI. This contribution is tax-deductible, and we will coordinate the transfer of property. Visit our web site for details on making a donation.

PURPLE HAIR CHALLENGE — Dye your hair purple during the month of May to raise CF awareness and challenge others to do the same. Similar in concept to the ALS ice bucket challenge, this fun – and visually pleasing – challenge raises awareness of cystic fibrosis and funds for CFRI's services.

DANCE LIKE A FOOL VIRTUAL DANCE PARTY — The third annual event was held last February in memory of CFRI staff member Danielle Mandella, with dozens of dancers from across the country logging in and dancing over a period of 6 hours. Join us in February: seek pledges and have fun while dancing and supporting CFRI's wellness programs – all from the comfort of your home.

CHARITABLE PLANNED GIVING — Planned giving offers benefits for donors that often include increased income and substantial tax savings, while providing the opportunity to meet your philanthropic goals and provide positive tax benefits.

HAVE AN IDEA? HOST YOUR OWN FUNDRAISER — Have fun, raise CF awareness and change lives. You could throw a virtual cocktail party, organize a virtual walk-a-thon, or come up with your own creative way to build strength and support for the CF community. Come up with an idea and we will support you!

For more information, please contact Stacie Reveles at sreveles@cfri.org.

CFRI Programs and Events

CFRI provides a range of services to meet the multi-faceted needs of our CF community.

CF Quality of Life (CFQoL) Financial Support for Individual Therapy

CFRI underwrites up to $125 per session for six sessions of counseling with the licensed therapist of one's choice. This nationwide service is available to children and adults with CF as well as to their immediate family members (siblings, spouses, partners, parents) until annual funds are expended.

Monthly Online Support Groups for the CF Community

For CF Caregivers

Third Tuesday of every month. Parents of children with CF meet at 5:00 pm PT. Parents and partners of adults with CF meet at 6:00 pm PT. Facilitated by a CF social worker, these groups provide peer-to-peer support to help families cope with the daily challenges of life with CF.

For Adults with CF

Third Monday of every month, 5:30 pm PT to 7:00 pm. Online Support Group for Adults with CF, which is open to participants nationwide and facilitated by a social worker well versed in issues facing adults with CF.

For Teens with CF

Third Wednesday of every month, 5:30 pm to 6:30 pm PT. This online Support Group for teenagers living with CF is facilitated by a CF social worker well versed in issues facing teenagers with CF. Parents need to give consent for their teenagers to attend.

For Those Who Are Bereaved – Navigating Grief to Growth

First Tuesday of every month, 5:00 to 6:30 pm PT. An online discussion and support group for those who have lost a loved one to CF, whether recently or in the past.

For Spanish-Speaking CF Community Members

Second Wednesday of every month, 5:00 to 6:30 pm PT. The group is open to Spanish-speaking adults with CF as well as family members of adults and children with CF. The group discussion is facilitated in Spanish by a medical social worker.

For Adults with CF Post-Transplant

Fourth Wednesday of every month, 5:00 pm to 6:30 pm PT. This group addresses the unique needs of those with CF who have received a double lung transplant and is open to post-transplant CF adults only. Facilitated by a social worker who lives with CF and is a lung transplant recipient.

For Adults with a Late CF Diagnosis

First Wednesday of every month, 5:00 pm to 6:30 pm PT. A discussion and support group for adults with CF who received a late diagnosis. The group is facilitated by two adults with late CF diagnoses.

CFRI Programs and Events

For Adults with CF Who Are Not Eligible for CFTR Modulators – *Launching Thursday, August 22*
Fourth Thursday of every month, 5:00 pm to 6:30 pm PT. A discussion and support group for adults with CF who are ineligible to or cannot use CFTR modulators. The group will be facilitated by a social worker who lives with CF.

Practical Mindfulness

Six-week online class taught by Dr. Julie Desch, offered twice a year. The class teaches a user-friendly and practical way to learn to meditate that easily carries over into daily life, tailored for the CF community.

CFRI's CFQoL Programs are generously supported by Viatris, Amgen, Gilead Sciences, Vertex Pharmaceuticals, and private donors.

Many Voices ~ One Voice CF Advocacy and Awareness Program

Our Advocacy and Awareness Program broadens understanding of the physical, emotional, and financial challenges faced by the CF community while advocating to reduce barriers to medical care and therapies and increase investment in research. We need your voice; please get involved!

Generously sponsored by Vertex Pharmaceuticals, Gilead Sciences, AbbVie, Genentech, Amgen, the EveryLife Foundation, and the Bucks County Cystic Fibrosis Alliance.

Faces of CF Diversity & Inclusion Program

CF impacts people of every race and ethnicity. This program advances awareness of our CF community's diversity, while creating resources – including podcasts and brochures – for underrepresented groups. Many of these resources are available in Spanish and Hindi.

Generously sponsored by Vertex Pharmaceuticals, Viatris, Gilead Sciences.

CFRI Programs and Events

CF Spring and Summer Retreats

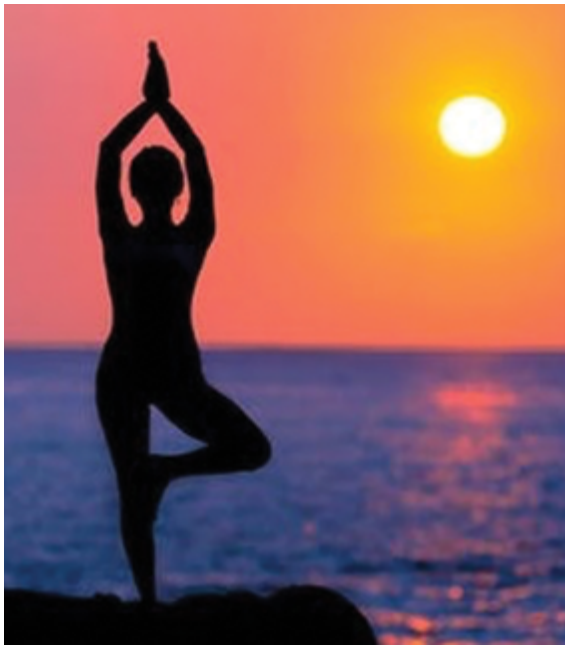
The annual CF Spring Retreat and CF Summer Retreat enhance education, positive coping skills, and social support for people who share common experiences with CF, and include educational presentations, exercise, arts and crafts, support groups, and much more. The 2024 Summer Retreat will be held at Vallombrosa Retreat Center in Menlo Park, CA from July 28 to August 1. ***Join us!***

Generously sponsored by AbbVie, Vertex Pharmaceuticals, Devin Wakefield, and private donors.

Embrace Retreat for Mothers of Children and Adults with CF

The Mothers Retreat provides peer support and expert speakers addressing CF-related resources, self-care for caregivers, stress reduction strategies, and other topics pertinent to coping with chronic illness. The retreat takes place on the first weekend of May in Menlo Park, CA.

Generously sponsored by AbbVie and Vertex Pharmaceuticals.



CF Wellness Initiative

The CF Wellness Initiative consists of three complementary multidisciplinary programs to help CF community members to achieve optimal physical and mental wellbeing. Components include Pilates, aerobics, Yoga, and CF Strength and Conditioning. Free online classes are ongoing.

Generously sponsored by Vertex Pharmaceuticals, Viatris, and contributions to CFRI's Dance Like A Fool event.

CFRI Programs and Events

CF Community Voices Video Podcast Series

Created by and for the CF community, CFRI's video podcast series is available on our Podbean and YouTube channels. Personal and professional CF experts address diverse topics including nutrition, financial planning, mental health, CF research, reproductive health, and more.

Generously sponsored by Viatris, Gilead Sciences and Vertex Pharmaceuticals.

A Breath of Fresh Air Gala Event

On Sunday, October 6, 2024, join us for our annual gala and support the search for a CF cure. The in-person event will be held at the breathtaking Nestldown Estate in Los Gatos, CA. In addition to inspiring stories, musical performances, gourmet food and local wines, we will honor our 2024 CFRI Champion.

Sponsored to date by Vertex Pharmaceuticals, AbbVie, Viatris, Heritage Bank, and GRAIL

For information about any of these programs, please call CFRI at 855.237.4669, email cfri@cfri.org, or go to www.cfri.org.

Purple Hair Challenge

Each May during CF Awareness Month, we challenge the community to dye their hair purple – the CF awareness color – with dye or using a phone app. Participants post their photos on social media with #purplehairchallenge, tag CFRI and challenge friends to join them.

Generously sponsored by Vertex Pharmaceuticals

CFRI Cystic Fibrosis Research Institute
RESEARCH · EDUCATION · ADVOCACY · SUPPORT

www.cfri.org | 855.cfri.now (237.4669)

The Cystic Fibrosis Research Institute was founded in 1975 as an independent 501(C)3 nonprofit organization by a group of family members whose children had cystic fibrosis. Our mission is to be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support. Our vision is to find a cure for cystic fibrosis while enhancing quality of life for the CF community.

We are able to provide our diverse programs and services thanks to our phenomenal volunteers, who generously share their time and expertise to advance research and improve the lives of those impacted by cystic fibrosis.