



July 28 – 30, 2023

Hope on the Horizon

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 28, 2023

Ms. Siri Vaeth, MSW, Executive Director
Cystic Fibrosis Research Institute
1731 Embarcadero Road, Suite 210
Palo Alto, California 94303-3339

Dear Ms. Vaeth,

It gives me great pleasure to welcome everyone to California's 16th Congressional District, the home of the Cystic Fibrosis Research Institute (CFRI), and to CFRI's 36th National Cystic Fibrosis Education Conference, "Hope on the Horizon."

Cystic fibrosis is a rare, life-threatening genetic disease in children and adults, affecting their respiratory, digestive and reproductive systems. The disease impacts individuals of every race and ethnicity. 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 cystic fibrosis community.

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

On behalf of the residents of the 16th Congressional District, I thank CFRI for organizing this event, and offer my best wishes for its success.

Warm regards,

A handwritten signature in black ink that reads "Anna Eshoo".

Anna G. Eshoo
Member of Congress



Welcome



Dear Friends,

Welcome to CFRI's 36th National Cystic Fibrosis Education Conference, *Hope on the Horizon*. 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 2023 conference provides you with the opportunity to hear from over 20 experts in the field of cystic fibrosis, addressing mRNA therapies, gene editing, phage therapy, pain management, cancer and other health screenings, paths to parenthood, health disparities, 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 2023 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

Hope on the Horizon – Conference Schedule

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

Friday, July 28, 2023

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

8:00 am – 8:45 am	Continental Breakfast Sequoia Room
8:50 am – 9:00 am	Welcome and Opening Remarks — Siri Vaeth, MSW, CFRI Executive Director Acacia Room Introduction of Research Presentation Emcee — Julie Desch, MD, Research Advisory Committee Chair
9:00 am – 9:45 am	Role of CFTR Arginine-933 in FDA-Approved Drug Potentiation Acacia Room — Stephen Aller, PhD
9:55 am – 10:40 am	Nanotechnology Toolkits for Cystic Fibrosis Gene Therapies Acacia Room — Steven Jonas, MD, PhD
10:50 am – 11:35 am	Development of Bacteriophage Therapy for Antimicrobial-Resistant Infections in Cystic Fibrosis — Paul Bollyky, MD, PhD Acacia Room
11:35 am – 12:30 pm	Lunch Break Sequoia Room
12:30 pm – 1:15 pm	Airway Stem Cell Transplantation into the Sinuses Using Fibrinogen Scaffold — Sriram Vaidyanathan, PhD Acacia Room
1:25 pm – 2:10 pm	Tolerance to Cell Permeable Antibiotics: Intracellular Adaptations of <i>Pseudomonas aeruginosa</i> — Naren Kumar, PhD Acacia Room
2:10 pm – 2:25 pm	Break
2:25 pm – 3:10 pm	Targeting IRBIT to Correct Bicarbonate Secretory Defects in Cystic Fibrosis — Zachary Sellers, MD, PhD Acacia Room
3:00 pm – 4:30 pm	Support Groups: Peninsula 1 – 4 — Adults with CF — Parents/Caregivers of Children with CF — Parents/Partners of Adults with CF — Adults Post Transplant
3:20 pm – 4:05 pm	Improving CF Airway Mucociliary Clearance: Toward Transition from Animals to Humans — Carlos Milla, MD; Nam Soo Joo, PhD Acacia Room
4:15 pm – 5:00 pm	Pathways Balancing Basal Mucin and CFTR-Mediated Fluid Secretion in the Human Distal Airway — Kenichi Okuda, MD, PhD Acacia Room
5:15 pm – 6:00 pm	In-Person Reception Dockside Room
6:00 pm – 6:45 pm	A Journey Through Rare: Because EVERYONE Deserves More Tomorrows Dockside Room — Rachel Alder

Saturday, July 29, 2023

- 7:30 am – 8:30 am **Continental Breakfast**
Sequoia Room
- 8:45 am – 9:00 am **Welcome and Opening Remarks**
Acacia Room — Siri Vaeth, MSW, CFRI Executive Director
— Introduce Emcee, Darrell Batchelder
- 9:00 am – 9:55 am **Phage Therapy** — Saima Aslam, MD, MS
Acacia Room
- 10:05 am – 11:00 am **All Hands on Deck to Cure Cystic Fibrosis** — Matthew Porteus, MD, PhD
Acacia Room
- 11:00 am – 11:15 am **Mini Break**
- 11:15 am – 12:10 pm **Understanding and Managing Pain in CF: A Biopsychosocial Approach**
Acacia Room — Deborah Friedman, PhD; Amanda S. Bruce, PhD
- 12:10 pm – 1:15 pm **Boxed Lunch Break**
Sequoia Room (Virtual: Optional Breakout Yoga/ Exhibitor Hall/Lounge)
- 1:15 pm – 2:10 pm **From Defining Health Disparities to Improving Health Equity in Cystic Fibrosis** — Susanna A. McColley, MD, FAAP, ATSF
Acacia Room
- 2:20 pm – 3:15 pm **Panel: Paths to Parenthood with CF** — Lucy Barnes, Matthew DeFina, Carl Robinson; moderated by Mary Helmers, RN
Acacia Room
- 3:15 pm – 3:30 pm **Mini Break**
- 3:30 pm – 4:25 pm **Advances in mRNA Therapy: New Applications for Cystic Fibrosis**
Acacia Room — Deepika Polineni, MD, MPH
- 4:25 pm – 5:30 pm **Exhibitor Hall / Break**
- 5:30 pm – 6:00 pm **Dinner Buffet (In-Person)**
Sequoia Room
- 6:00 pm – 7:15 pm **CFRI Awards Celebration with Special Guests**
Sequoia Room
- 7:30 pm – 9:30 pm **Dance Party (In-Person)**
Dockside Room

Sunday, July 30, 2023

- 8:00 am – 9:00 am **Continental Breakfast**
Sequoia Room
- 9:00 am – 9:15 am **CFRI Overview** — Siri Vaeth, MSW, CFRI Executive Director
Acacia Room
- 9:15 am – 10:05 am **Embracing the Future: Aging with CF** — Ahmet Uluer, DO, MPH
Acacia Room **Colon Cancer and Cystic Fibrosis: My Lived Experience** — Anna Payne
- 10:15 am – 11:05 am **Advances in Stem Cell Research for the Treatment of CF**
Acacia Room — Brigitte Gomperts, MD
- 11:05 am – 11:20 am **Break**
- 11:20 am – 12:15 pm **Turning Struggles Into Strengths** — Alanah Rosenbloom, MSW
Acacia Room
- 12:15 pm – 12:30 pm **Closing Remarks** — Siri Vaeth, MSW, CFRI Executive Director
Acacia Room

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!"

A screenshot of the virtual conference login screen. At the top is the CFRI Cystic Fibrosis Research Institute logo. Below it is a form with two input fields: 'Username or Email Address' and 'Password'. There is a 'Remember Me' checkbox and a 'Log In' button. An eye icon is next to the password field to toggle visibility.

Main Lobby



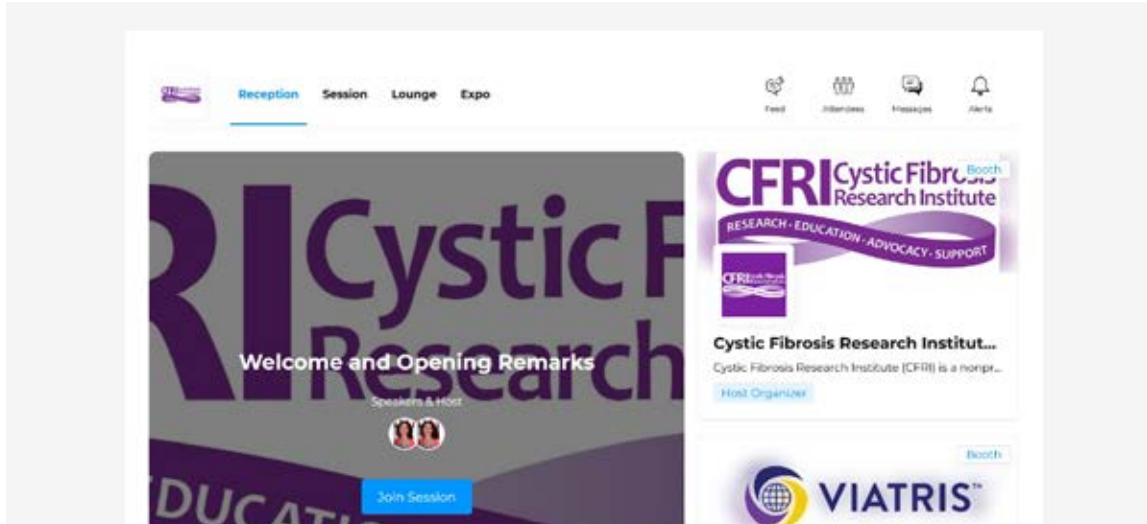
In the main lobby, attendees may **use the menu at the top of the screen** to access the Auditorium (watch streamed sessions), Exhibit Hall (visit the virtual booths), and the Lounge (to Video Chat with other attendees). From the menu attendees can also watch the welcome video, view the Conference Agenda,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

Tips for Navigating the Virtual Conference

Reception Area

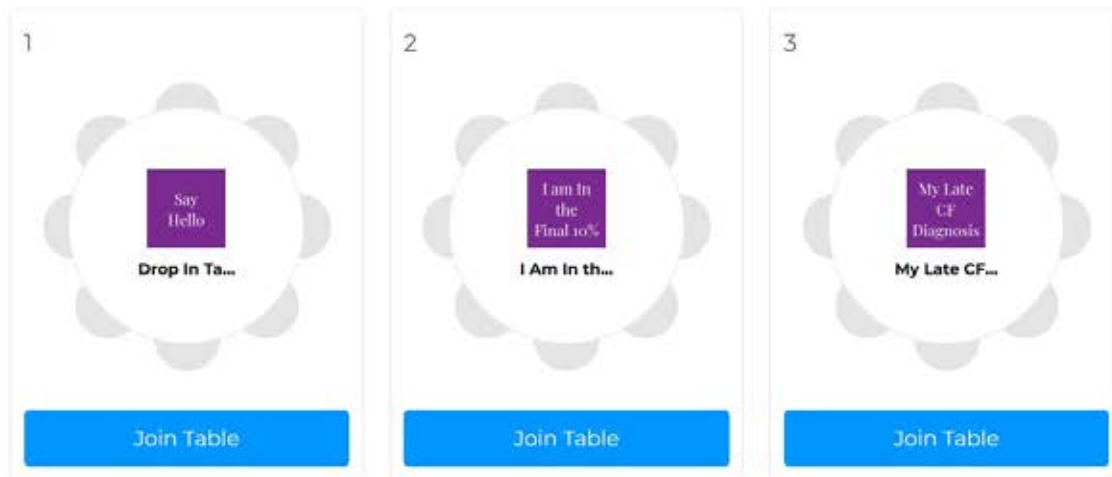
The Reception Area can also be accessed from the menu at the top of the screen. This is where you will see what session is in progress, or soon to begin. As you enter the Reception Area, please allow a few moments for the content to load.

CFRI – Cystic Fibrosis Research Institute Hope on the Horizon 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 conference attendees – whether or not you 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 48 hours prior to arriving at the conference to confirm that they are negative for COVID-19.
2. Everyone in attendance is strongly 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. Your nametag ensures 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 think you have COVID-19, a cold, virus or the flu, you must leave the conference. The conference is available to view live on our virtual platform, and recordings will be available to view online after the event should you miss the conference.
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, 2023.
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 from a CFF-accredited laboratory 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. All meals will be served by hotel personnel to those with 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 36th National CF Education Conference

Premiere Sponsors — *Viatis; Vertex Pharmaceuticals*

Sustaining Sponsor — *Genentech*

Diamond Sponsors — *Gilead Sciences; Chiesi USA*

Platinum Sponsors — *AbbVie; Boomer Esiason Foundation*

Bronze Exhibitors — *Alcresta Therapeutics; Digestive Care, Inc.;*
Foundation Care, An AcariaHealth Solution; NeilMed Pharmaceuticals;
Walgreens Specialty Pharmacy; 4DMT; First Wave BioPharma

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

Supporter — *Prodigy Press, Inc.*

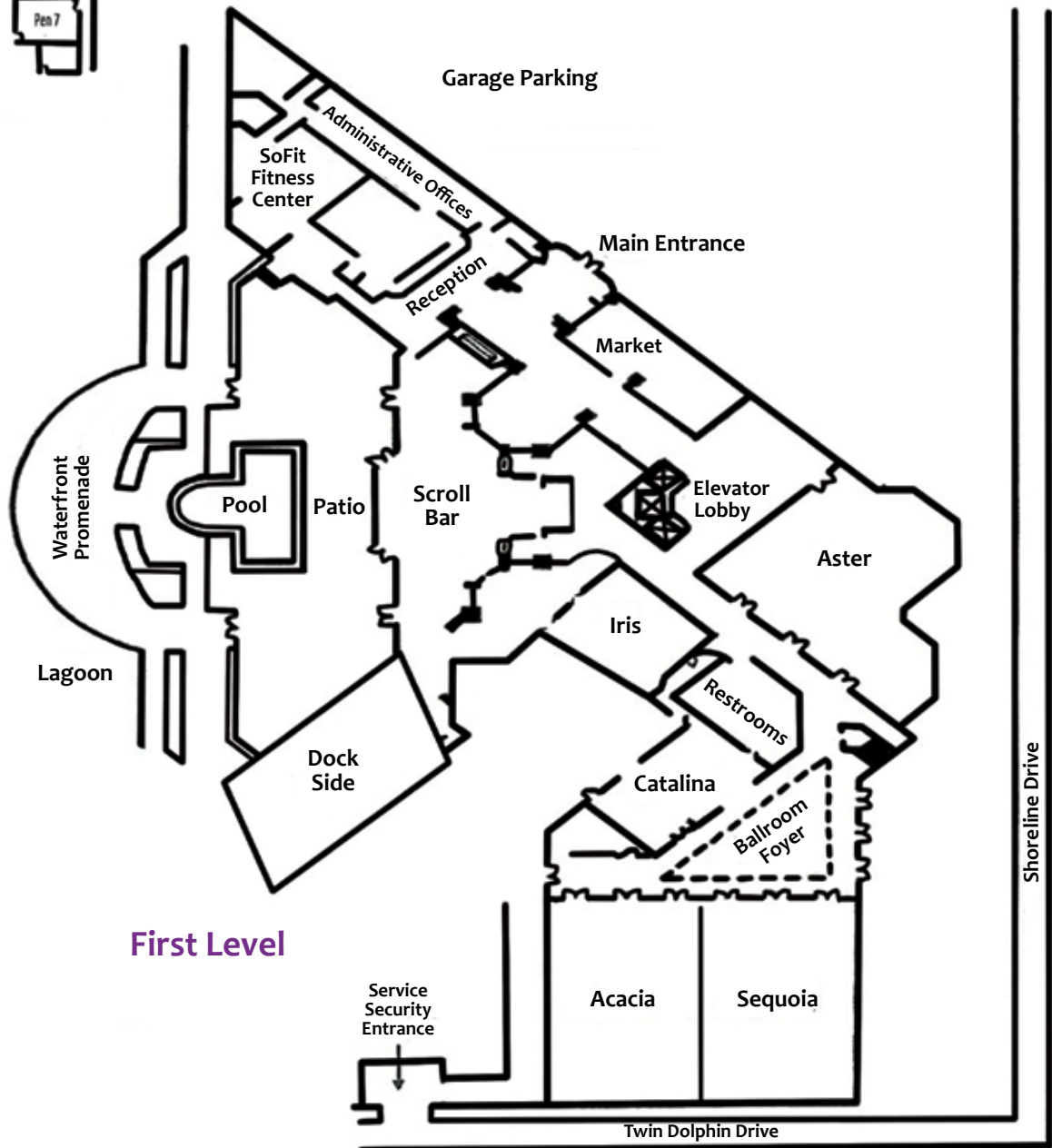
List current as of 07/3/2023. Updates to list available in digital program.



Grand Bay Hotel San Francisco

223 Twin Dolphin Drive
Redwood City, CA 94065

1 650 598-9000



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 is currently in his fifth two-year term serving on the West Valley/Mission College Citizens Bond Oversight Committee. He served ten years as a Director of Neighborhood Housing Services Silicon Valley, and eight years with 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 28, and her son Dylan is 24. She lives in Santa Cruz, California.



Julie Desch, MD — Friday Emcee

At 62 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. Her current online offering, "Zoom Into Now," is a monthly drop-in mindfulness session for the CF community. Julie serves on the Board of Directors of CFRI and serves as Chair of CFRI's Research Advisory Committee.



Darrell Batchelder — Saturday Emcee

Darrell Batchelder has been involved with CFRI since 1990, when he and his wife Darlene's youngest son Joe was diagnosed with CF. Joe is healthy and working full time today, thanks to the advancements made in treatment protocol and drugs created, at least in part, from research funded by generous donations to CFRI. Darrell has served as our Conference Emcee in the past and prior to retiring, he owned a Silicon Valley Ad Agency.

Speaker Profiles

*denotes CFRI-funded researchers



Rachel (Rae) Alder

Salt Lake City, Utah

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 regards 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.



Stephen G. Aller, PhD *

University of Alabama / Birmingham, AL

Dr. Stephen Aller is an Associate Professor at the Department of Pharmacology and Toxicology at the University of Birmingham in Alabama (UAB). In 1988, he studied regulation, molecular biology, and electrophysiology of CFTR from the shark *Squalus acanthias* compared to the human channel as a Hearst Foundation High School Research Scholar at the Mount Desert Island Biological Laboratory (Bar Harbor, Maine). In his Master's Degree (MS) thesis research, he cloned and characterized the first guanylyl cyclase receptor (NPR-B) and showed activation of CFTR-dependent chloride conductance through cGMP-mediated phosphorylation. As part of his PhD thesis

research, he received training in expression, purification and protein crystallization, and solved the structure of a human metal uptake transporter by cryo-electron microscopy (cryo-EM). This work led to an NIH-postdoctoral fellowship at the Scripps Research Institute, where he established overexpression, purification, and crystallization protocols and solved the X-ray crystal structure of the mammalian ABC protein P-glycoprotein (ABCB1), a homolog of CFTR (ABCC7).

His lab is currently studying the structure-function of CFTR by single-particle cryo-EM. He established key collaborations at UAB in this regard, including work with Drs. Steven Rowe and Wei Wang to challenge the currently accepted binding location of potentiator drugs to CFTR by patch clamp and cryo-EM. He is also a major user on UAB's newly installed ThermoFisher Glacios 200kV electron microscope for cryo-EM which was funded by a successful NIH S10 Award (S10OD024978) to which he contributed major grant writing and a hands-on 3-day in-person data processing workshop held on the UAB campus. He has two extremely talented trainees in the laboratory who have contributed the micrographs and classification of CFTR R933 mutants for the CFRI Conference.

Speaker Profiles



Saima Aslam, MD, MS

University of California / San Diego, CA

Dr. Aslam is a Professor of Medicine in the Division of Infectious Diseases and Global Public Health, and Director of the Solid Organ Transplant Infectious Diseases Service at the University of California San Diego. Dr. Aslam has been actively engaged in clinical research as well as development of clinical practice guidelines regarding within the field of transplantation. She is a founding member of the Center of Innovative Phage Applications and Therapeutics (IPATH), the first clinical phage therapy center in the US and has extensive experience of treating patients with phage therapy for treatment of antibiotic recalcitrant infections in the US. She is the PI of a pilot grant from the Cystic Fibrosis Foundation to develop a registry and associated phage library for people with cystic fibrosis infected with *Burkholderia* species. She is also the site PI of several phage related multicenter clinical trials including patients with *S. aureus* bacteremia and prosthetic joint infection. Additionally, she serves as the site PI for two U01 funded (NIAID) clinical trials aimed at comparing outcomes of liver and kidney transplantation from HIV-infected and uninfected deceased donors into HIV-infected transplant recipients; as well as a third trial aimed at assessing response to COVID-19 vaccination in liver and kidney transplant recipients (NIAID).



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 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.



Amanda Bruce, PhD

University of Kansas / Kansas City, KS

Dr. Amanda Bruce is an Associate Professor and licensed clinical psychologist at the University of Kansas Medical Center (KUMC). She did her undergraduate work at the University of Kansas and her doctoral training at Pennsylvania State University. Her internship was at the Boston Consortium VA associated with Harvard Medical School. She has been faculty at KUMC since 2011, and she started working with adults with cystic fibrosis in 2016. She has 80 peer-reviewed publications related to health behaviors and decision-making, treatment adherence in chronic diseases, and psychological interventions. She has received funding from the National Institutes of Health, the United States

Department of Agriculture, and the Cystic Fibrosis Foundation.

Speaker Profiles



Deborah Friedman, PhD

Massachusetts General Hospital / Boston, MA

Dr. Deborah Friedman is a licensed clinical psychologist in the Massachusetts General Hospital (MGH) Department of Psychiatry, and Assistant Professor of Psychology (Psychiatry) at Harvard Medical School. She is co-director of the MGH Pediatric Behavioral Medicine Program, providing clinical, research, and training activities focused on improving health, resilience and coping with acute and chronic illness in children, adolescents, and their families. She has been a member of the MGH Pediatric and Adult CF Programs' interdisciplinary team, providing direct clinical care to people with CF for over 10 years.

Dr. Friedman's research focuses on developing mental health and palliative care interventions that can be integrated into CF team-based care to improve the lives of people with CF and their families. She has authored an 8-session cognitive behavioral therapy-based program for the prevention and treatment of depression and anxiety for adults coping with CF (CF-CBT) as well as a developmentally targeted adaptation for adolescents with CF, with resources for caregivers (CF-CBT-A). Both interventions were developed with input from the CF community and CF healthcare providers to specifically address the emotional challenges that people with CF face. Following a multi-center randomized controlled trial of CF-CBT, an implementation trial in conjunction with the CF Foundation (CFF) is now underway with the aim to disseminate CF-CBT across CF centers in the U.S. and Canada, providing a model to increase access to an evidence-based mental health intervention for people with CF.

A therapist-guided internet-delivered version (eHealth CF-CBT) has also been developed in English and Dutch and is currently being disseminated across CF centers in the Netherlands (Marieke Verkleij, PI) with support from the Dutch CF Foundation and Vertex Pharmaceuticals. Dr. Friedman is also co-principal investigator with Dr. Amanda Bruce of a CFF-funded clinical pilot and feasibility award to engage key stakeholders in the development and pilot of a brief, telehealth, CF-specific psychosocial pain management intervention.



Brigitte Gomperts, MD

University of California / Los Angeles, CA

Dr. Gomperts is a physician-scientist who has been a faculty member at the University of California Los Angeles (UCLA) for over 19 years. She is Professor of Pediatrics and Pulmonary Medicine and Associate Director for Translation for the UCLA Broad Stem Cell Research Center. She is also Co-Director of the Jonsson Comprehensive Cancer Center Cancer Stem Cell Biology Program and Vice Chief of Research for Pediatric Hematology-Oncology. Her lab studies repair and regeneration of the lungs, and how the normal repair mechanisms are altered in lung diseases. She is particularly interested in stem/progenitor cell populations in the lungs and determining the regeneration potential

of each of these subpopulations in order to better understand repair and the possibility of cell-based therapies for lung diseases. Her lab is using novel models to understand repair and regeneration in the proximal and distal lung, and has expertise in mouse models of lung diseases and induced pluripotent stem cell lung disease modeling. Dr. Gomperts' major areas of interest include lung fibrosis, mucociliary clearance and cystic fibrosis. As a physician-scientist, her lab is particularly interested in translational research that will result in new therapies for lung diseases.

Speaker Profiles



Steven J. Jonas, MD, PhD *

University of California Los Angeles / Los Angeles, CA

Dr. Steven Jonas is a pediatric physician-scientist and Assistant Professor in the Department of Pediatrics at the University of California Los Angeles (UCLA) David Geffen School of Medicine and California NanoSystems Institute. He is a member of the UCLA Eli & Edythe Broad Center of Regenerative Medicine & Stem Cell Research and the Jonsson Comprehensive Cancer Center. His multidisciplinary research team targets the development and application of new technologies and methods to support the children's health and regenerative medicine research communities in accelerating the discovery and implementation of emerging gene & cellular therapeutic approaches

and precision medicine-based diagnostic tools.

A primary focus of this research explores strategies for improving how gene therapies are manufactured through the design of nanotechnologies that enable rapid, safe, cost-effective, and efficient delivery of genes and genome-editing machinery. These capabilities motivate the Jonas group's efforts to create tools that enable stem cell biologists to probe and to interact with stem cells more precisely and empower clinical scientists to apply this knowledge to design and implement new therapies more rapidly and broadly.

Dr. Jonas' research program is supported through a NIH Director's Early Independence Award and additional funding provided by the Cystic Fibrosis Foundation and from a CFRI New Horizons Award. He is a Young Investigator awardee of the Alex's Lemonade Stand Foundation for Childhood Cancer Research, the Hyundai Hope on Wheels Foundation, and the Tower Cancer Research Foundation.



Nam Soo Joo, PhD *

Stanford University / Palo Alto, CA

Dr. Joo has extensive experience in cystic fibrosis research with a special emphasis on a potential role played by airway submucosal glands in the process of CF lung disease. Dr. Joo completed his undergraduate studies in pharmacology at Sung Kyun Kwan University in Seoul, Korea. After earning an MA and PhD in pharmacology from the University of Missouri in Columbia in 1999, he joined the Cystic Fibrosis Research Laboratory, directed by Dr. Jeffrey Wine, at Stanford. 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; and a mucociliary clearance (MCC) assay for measuring a mucociliary clearance velocity. With these methodologies, defects were discovered in submucosal gland secretory functions in CF airways from human and animal CF models (CF pigs and CF ferrets).

Speaker Profiles



Naren Gajenthra Kumar, PhD *

University of California / Berkeley, CA

Dr. Naren G Kumar received his PhD in Microbiology and Immunology at Virginia Commonwealth University in 2020. His doctoral research characterized lipid-degrading enzymes produced by *Staphylococcus aureus* and their role in bacterial biofilm formation and host lipid responses to injury and infection. Interested in further understanding how the interaction of bacteria with the host can influence persistence at the site of infection, he joined the lab of Dr. Suzanne Fleiszig at the University of California Berkeley.

His current research in the Fleiszig lab aims to understand how a novel population of the bacteria *Pseudomonas aeruginosa* persists inside cells and tolerates treatment with antibiotics. Using live imaging to study the dynamics of bacterial gene expression, replication, and sensitivity to antibiotics in infected cells, he hopes to understand why some populations of intracellular bacteria are less sensitive to antibiotic treatment. Having witnessed first-hand the challenges of treating a complicated *pseudomonas* infection of a close family member, he strives to improve the understanding of persistent bacterial infections to identify novel therapeutic targets to manage and treat challenging bacterial infections.



Susanna A. McColley, MD, FAAP, ATSF

Ann & Robert H. Lurie Children's Hospital of Chicago / Chicago, IL

Susanna McColley, MD, FAAP, ATSF is a pediatric pulmonologist whose research focuses on improving the health of people with cystic fibrosis through understanding risk factors for more severe disease, improving methods for screening and diagnosis, describing health disparities, and testing new treatments. She is currently leading a multidisciplinary team of clinicians, community members and public health professionals to improve timeliness and equity of CF newborn screening. As director of an NIH-funded training program, she is passionate about supporting the next generation of child health researchers, especially those currently underrepresented in the biomedical research workforce.

Dr. McColley serves as the Scientific Director for Interdisciplinary Research Partnerships at Stanley Manne Children's Research Institute, Ann & Robert H. Lurie Children's Hospital of Chicago; Professor of Pediatrics in Pulmonary and Sleep Medicine at Northwestern University Feinberg School of Medicine; Associate Clinical Director for Child Health and Director of the TL1 Multidisciplinary Program in Child and Adolescent Health at Northwestern University Clinical and Translational Sciences Institute; and Editor-in-Chief for Pediatric Pulmonology. Her research is funded by the Cystic Fibrosis Foundation, the National Institutes of Health, the Center for Disease Control and Prevention, and The Legacy of Angels Foundation.

Speaker Profiles

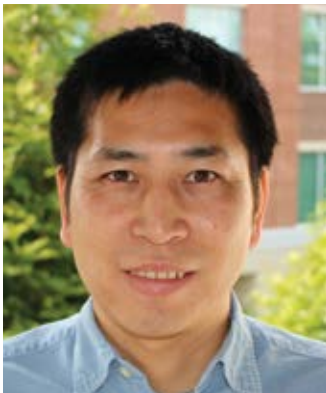


Carlos Milla, MD *

Stanford University / Palo Alto, CA

Carlos Milla is Professor of Pediatrics and (by courtesy) of Medicine at Stanford University School of Medicine, where he is also Associate Director for Translational Research at the Center for Excellence in Pulmonary Biology at Stanford. Dr. Milla is also the Director of the Stanford Cystic Fibrosis Center and the CF Therapeutics Development research Program. He has actively participated in multiple CF clinical research studies and has accumulated substantial experience on the development of novel outcomes in CF. This includes participation in multiple clinical trials, from early phase to pivotal trials, as well as participating in multiple advisory boards for drug development focused on CF and other airway disorders. Dr. Milla has published and

lectured extensively on the topics of cystic fibrosis and the genetics of rare lung diseases. Current areas of research include early CF lung disease development and the pathophysiologic mechanisms involved in the defective mucociliary clearance characteristic of CF. Additional research interests include active programs for biomarker discovery for chronic pulmonary conditions such as primary ciliary dyskinesia, pulmonary hypertension, chronic lung disease of infancy and interstitial lung disease.



Kenichi Okuda, MD, PhD *

University of North Carolina / Chapel Hill, NC

Dr. Okuda obtained his MD degree from Yamagata University in Japan, followed by residency training in internal medicine and fellowship in respiratory medicine. Thereafter, he entered the graduate school of medicine at the University of Tokyo where he learned advanced techniques in molecular biology, as well as tissue culture and mouse model management. In the second year of graduate school, he applied for postdoctoral training in Dr. Richard Boucher's laboratory at University of North Carolina at Chapel Hill to engage in studies of airway and mucus biology.

Under Dr. Boucher's supervision, Dr. Okuda successfully characterized the regional expression patterns of major airway secretory mucins, MUC5AC/MUC5B, and CFTR/ionocytes in normal and CF human airways. Using this study, he earned his PhD degree in Medicine from The University of Tokyo in Japan. Within these works, he developed microdissection techniques for small airway tissue isolation and in vitro small airway epithelial cell culture model comparable to large airways, which has been utilized in the CFRI-funded project. Dr. Okuda's overall research interest focuses on how the mucociliary clearance (MCC) system is regulated to maintain homeostasis in the lung and how it fails in muco-obstructive lung diseases, including CF. Based on these research interests, his long-term career goal is to work, as a professional investigator, toward a full understanding of the MCC system in the lungs and contribute to the improvement of the prognosis in all patients with muco-obstructive lung diseases.

Speaker Profiles



Anna Payne

Langhorne, PA

Anna Payne is a 36-year-old woman with CF who is battling stage-4 colon cancer. She lives in Middletown Township, in Bucks County, PA, where she is a dedicated public servant and patient advocate. She works as an administrative assistant for Bucks County Commissioner Diane Marseglia. She is also an elected official serving as the Chair of the Board of Supervisors in Middletown Township. She serves as Vice-chair on the Pennsylvania Rare Disease Advisory Council. She founded a non-profit organization, the Bucks County CF Alliance, with the mission to help CF stand for “cure found” and raise awareness about the link between CF and colon cancer.



Deepika Polineni, MD, MPH

Washington University / Saint Louis, MO

Deepika Polineni completed her residency in Internal Medicine and fellowship in Pulmonary Medicine with an emphasis on cystic fibrosis (CF) care and translational research. She joined Washington University in St. Louis in 2022, where she serves as the Cystic Fibrosis Center Director and is an Associate Professor in the Department of Pediatrics, Division of Allergy and Pulmonary Medicine. Her research program focuses on the identification of non-CFTR genetic modifiers of CF lung disease using human airway transcriptomics and metabolomics to identify novel gene targets, and airway cellular models to study mechanisms of influence.

These ‘omics translational studies are complementary to the efforts of the International CF Modifiers Consortium with the goal of advancing personalized therapies in CF.

Dr. Polineni has served as a lead principal investigator in international clinical trials of CFTR modulators and supported a diverse body of CF research as a site-level clinical trial investigator. She serves on the Medical Advisory Committee and the Research Advisory Committee for the Cystic Fibrosis Research Institute. She additionally co-chairs the Preclinical/Clinical working group of the Cystic Fibrosis Foundation Genetic Therapies Working Group. These efforts are aimed at supporting care for people with CF and advancing new therapies, particularly mutation agnostic nucleic acid based treatments, with the goal of reducing health disparity gaps and serving a global CF community.



Matthew Porteus, MD, PhD

Stanford University / Palo Alto, CA

Matthew Porteus MD, PhD, is the Sutardja Chuk Professor of 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. He is the Director of the Stanford Center for Definitive and Curative Medicine and the co-Executive Director of the Stanford Cell and Gene Therapy GMP Facility called the Laboratory for Cell and Gene Medicine. His primary research focus is on developing genome editing as an approach to cure disease, particularly those of the blood (most notably 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

Speaker Profiles

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 under going 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. He has been a scientific founder of CRISPR Tx and a founder of Graphite Bio and serves on several SAB's. He is a strong advocate for assuring that the next generation of transformative medicines reaches the global community in partnership with those communities.



Alanah Rosenbloom, MSW

San Jose, CA

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.



Zachary Sellers, MD, PhD *

Stanford University / Palo Alto, CA

Dr. Sellers is an Assistant Professor of Pediatrics and the Associate Chair of Research in Pediatric Gastroenterology at Stanford University. He is also the Director of the Stanford Children's Pancreas Program, where he specializes in the clinical care of children with cystic fibrosis and other pancreatic diseases. Dr. Sellers' research is centered around improving the gastrointestinal, pancreatic, and liver health of those with cystic fibrosis. His laboratory investigates new pharmacologic and gene editing approaches to correct ion transport defects in cystic fibrosis, with a special focus on bicarbonate transport. He also studies the pathophysiologic interactions between the pancreas and intestine in pancreatic and intestinal diseases. Additionally, Dr. Sellers is an active

clinical researcher in pediatric pancreatitis and cystic fibrosis-associated liver disease. His research is funded by the NIH, CF Foundation, CF Research Institute, and Stanford School of Medicine. He is a prior CFF DIGEST awardee and past recipient of the CFF's LeRoy Matthews Physician-Scientist Award.

Speakers Profiles



Ahmet Uluer, DO, MPH

Boston Children's Hospital/ Brigham & Women's Hospital / Boston, MA

Dr. Ahmet Uluer is a medicine and pediatric trained pulmonologist and Director of the Adult Cystic Fibrosis Program at the combined Boston Children's Hospital and Brigham & Women's Hospital Adult Cystic Fibrosis Center. He is also Director of the local Therapeutic Development Network (TDN) and oversees a highly effective team of Research Coordinators and Assistants to conduct corporate sponsored and investigator-initiated clinical trials, along with two phase 2 trials as national PI. He is Co-Chair of the Protocol Review Committee of the TDN.

His clinical and research interests involve all aspects of cystic fibrosis care, including quality improvement initiatives, transitional care and outcomes research. He has been interested in complications related to acute and chronic pulmonary therapies in an aging CF population, including kidney disease and hearing loss. Dr. Uluer completed a translational research project while earning his Master's in Public Health degree at the Harvard TH Chan School of Public Health, looking at urinary biomarkers associated with kidney injury, as well as a prospective study of monitoring hearing loss utilizing point of care hearing assessment. He is Director of the Weitzman Family Bridges Adult Transition Program at Boston Children's Hospital, providing age-appropriate care and transitional care support to all adult survivors with congenital or pediatric acquired chronic illness. He is working on transitional care processes and outcome measures for those with childhood-onset chronic diseases and along with other collaborators, working on building the capacity, from many perspectives, for them to be cared for in an adult medical home.



Sriram Vaidyanathan, PhD *

Nationwide Children's Hospital / Columbus, OH

Sriram Vaidyanathan, PhD, is a principal investigator in the Center for Gene Therapy at the Abigail Wexner Research Institute at Nationwide Children's Hospital and an assistant professor of Pediatrics at The Ohio State University College of Medicine. He earned his Bachelor's degree in Biomedical Engineering from Purdue University and his PhD in Biomedical Engineering from the University of Michigan, Ann Arbor. He completed his postdoctoral training with Dr. Matthew Porteus at Stanford University, CA. His primary research interest has been the development of gene and cell therapies. His most recent work has focused on the development of an autologous gene corrected airway stem cell therapy to treat CF sinus disease. His lab continues to further

develop genome editing approaches to treat CF and other airway diseases.

Panelist Profiles



Lucy Barnes

Ashland, OR

Lucy Barnes is a 34-year-old woman with CF who lives in Ashland, Oregon. She is the mother of two sons and works as a professional birth and postpartum doula. Lucy received her BA in Psychology from the University of California Santa Barbara. She loves the outdoors and on the weekends enjoys going to see live music, tending the garden, hot yoga, and wrangling her backyard chickens.



Matthew De Fina

Napa, CA

Matt De Fina was born in 1977 and diagnosed with CF at 20 days old. His parents were initially told that he would not live past his 4th birthday. Through a dedicated, determined, and disciplined approach, Matt and his parents were able to beat that prognosis as he went on to enjoy a normal childhood by participating in many sports, doing well in school, and becoming blessed with many friendships. Matt went on to earn both a Bachelor's of Science degree and a Master's Degree in Business Administration. In his early 30's, Matt's lung function declined to a point at which he required a double-lung transplant to survive. He received that transplant in 2012 at Stanford Hospital. However, those lungs ultimately developed chronic rejection, and in July of 2020, he underwent a successful second double-lung transplant. Matthew, now 45 years old, is thriving, and his lung function is the highest it's ever been in his adult life. Matthew coaches high school volleyball, owns and operates a successful winery in Napa Valley, and works full-time as a Joint Replacement Representative for Stryker Orthopaedics. He and his wife Denise have been married for 20 years and have a beautiful 15 year-old daughter, Gracie, who is a freshman in high school.



Carl Robinson

San Jose, CA

Carl Robinson is a 45-year-old father of two who has worked in technology for over three decades. He is still working full-time and manages multiple people around the globe. His two daughters, aged six and 20 months, keep him grounded and focused on the essential things. Carl helps out his wife as much as he can, but he has no idea how she keeps up with them and their loving chaos.

His CF life started at Stanford in Palo Alto when he was born, where he still gets care. Carl uses all the technology to improve his health and work/life balance. From managing his diabetes to airway clearance, technology has improved over the years. Any spare time he has gets used up by tending to his bee hives, doing home improvement projects, playing computer games, having fun with his family, and helping his mom Ann Robinson age gracefully.

Support/Discussion Group Facilitators

Patricia Delfino, MSW, LCSW

Ms. Delfino has worked in counseling services since her graduation from Virginia Commonwealth University in 1981 with a Master's in Psychiatric Social Work. She has worked in outpatient mental health and in-patient programs, as well as in home health. In this capacity she assists patients and their families with crisis intervention counseling, focusing on acceptance and management of new diagnoses. Ms. Delfino has extensive experience providing counseling for children and teens with chronic and acute mental health issues. She has two adult sons, Brian, who does not have CF, and Brett, who has CF.



Sonya Haggett, LCSW

Sonya is a licensed clinical social worker from the San Francisco Bay Area living with cystic fibrosis, who is six 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 Summer Retreat committee member. Her clinical practice has focused on community mental health where medical, criminal justice, and aging issues intersect. She is facilitating CFRI's monthly support groups for adults with CF post-transplant.



Deborah Menet, LCSW

Debbie Menet, LCSW, is a Licensed Clinical Social Worker in the Cystic Fibrosis Center at Lucile Packard Children's Hospital – Stanford. She has over 20 years of experience working with children, adolescents and families in both school-based and medical settings. Currently Debbie provides support to children living with CF and their families, including providing individual and family therapy. She has presented nationally to parents of children with chronic illness and has facilitated groups for both teens and young adults with chronic illness, including co-facilitating the CFRI Teen Support group. Debbie likes to consider new and innovative ways to meet the needs of the children, teens, and families with whom she works. Debbie is currently completing a yoga teacher training, and enjoys being active outdoors in beautiful Northern California!



Kate Yablonsky, LCSW

Kate Yablonsky is a licensed clinical social worker with over 10 years of experience in medical social work with chronic illness populations. She spent the first decade of her career at Lucile Packard Children's Hospital Stanford working with pediatric oncology patients and their families. In 2018, she transitioned over to "Big Stanford," to begin working with adults with pulmonary disease (cystic fibrosis and interstitial lung disease). Kate has a passion for group work, and has facilitated many kinds of groups over the years, including a parent support group for stem cell transplant families, support groups for school-aged children and teenagers with cancer, support groups for bereaved siblings of children with cancer, and most recently, a group for adult patients living with pulmonary fibrosis and – through CFRI - groups for caregivers of children and adults with CF.

Panel Moderator



Mary Helmers, RN, BSN

Mary earned her Bachelor of Science degree from the University of San Francisco. Before moving to Lucile Packard Children's Hospital (LPCH) at Stanford she worked at the Veterans' Affairs Medical Center in San Francisco as a staff nurse with adult medical, surgical, cardiothoracic and neurosurgery patients. Mary's passion was pediatrics, and in 1985 she came to LPCH, where she worked as a staff nurse specializing in pediatrics in various hospital departments.

Mary's area of interest was pulmonary medicine and in particular, taking care of patients with cystic fibrosis. Beginning in 1992, Mary moved to the outpatient clinics and worked as the Allergy-Immunology/CF Nurse Coordinator for two years before starting a family. As a relief nurse for the clinics, she continued her interest in pulmonary medicine, covering and working with the CF and Allergy Nurse Coordinators. She took the role as the Adult Cystic Fibrosis Nurse Coordinator in June 2000. In October 2008, she transferred to her current job as the Pediatric CF Nurse Coordinator. In addition to being honored by Lucile Packard Children's Hospital at Stanford, Mary was recognized as CFRI's CF Professional of the Year in 2004, and CFRI's Cystic Fibrosis Champion in 2020.



2023 CFRI Awards and Awardees



The 2023 CFRI CF Professional of the Year Award

Deepika Polineni, MD, MPH

Deepika Polineni is Director of the Cystic Fibrosis Center and an Associate Professor of Pediatrics at Washington University in St. Louis, MO. Her research program focuses on the identification of non-CFTR genetic modifiers of CF lung disease using human airway transcriptomics and metabolomics to identify novel gene targets, and airway cellular models to study mechanisms of influence. The goal of her research is advancing personalized therapies in CF. Dr. Polineni has served as a lead principal investigator in international clinical trials of CFTR modulators and has supported a diverse body of CF research as a site-level clinical trial investigator. These efforts are aimed at improving care for people with CF and advancing new therapies, particularly mutation-agnostic nucleic acid-based treatments, with the goal of reducing health disparity gaps and serving a global CF community. Dr. Polineni gives generously of her time to support the broader CF community, including serving on CFRI's Medical Advisory Committee and Research Advisory Committee.



The 2023 David Stuckert Memorial Volunteer of the Year Award

Zoe Davies, RN, PNP

Zoe Davies is a Pediatric Nurse Practitioner who until earlier this year spent nearly three decades working as a pivotal member CF Research Team at the Stanford Center for Excellence in Pulmonary Biology. Zoe was incredibly passionate about teaching, supporting, and providing the best care to patients who volunteered to help advance therapies – and the search for a cure – for CF. Zoe always understood that CF impacts both the patient and the entire family and her relationships with study subjects and family members were close, as she protected their interests and rights. She earned the trust and affection of the Stanford CF community—colleagues, patients, families, friends—over three decades that transformed CF. She translated loss and grief into new determination to improve lives. For years, Zoe has been a dedicated volunteer with CFRI, where she serves as Secretary of the Board of Directors and on the CFRI Community Newsletter Committee.

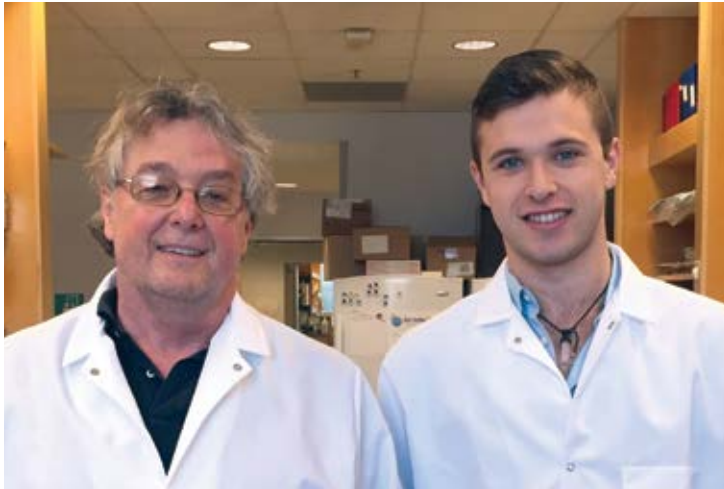


The 2023 CFRI Partners in Living Award in Memory of Anabel and Isabel Stenzel

Scott Pinner, MD

Dr. Scott Pinner is a husband, father, athlete, and a Physical Medicine and Rehabilitation physician who works with musculoskeletal and spinal cord injury patients and stroke survivors. Living with cystic fibrosis, as a youth Scott ran cross country and played soccer and tennis, which helped to maintain his lung function. Scott's health declined in his late thirties, after the rigors of medical school, residency, and launching his medical career. He was listed for transplant, but prior to receiving this, he endured colon cancer with serious complications and numerous pneumothorax. Scott received a life-saving double lung transplant in 2014. Since his transplant, Scott has become a strong advocate for organ donation, and an athlete in the Transplant Games. He has been a member of the CFRI community for many years, and currently serves on CFRI's Research Advisory Council. Scott embodies the spirit of this award and Anabel and Isabel Stenzel's memory.

2023 CFRI Awards and Awardees



Dieter Gruenert and his son in his lab

The 2023 Paul M. Quinton Cystic Fibrosis Research Legacy Award

Dieter Gruenert, PhD
(Posthumous award)

Dieter Gruenert was a geneticist in the Department of Otolaryngology-Head and Neck Surgery at the University of California San Francisco (UCSF). He originally joined the faculty at UCSF in 1986, where he developed many of the human cystic fibrosis (CF) and non-CF airway epithelial cell lines used in airway disease research

throughout the world. Dr. Gruenert can be considered the pioneer of gene editing; he developed a prototype of targeted genome editing called Small Fragment Homologous Replacement (SFHR). This technique paved the way to the extraordinary and more efficient CRISPR/Cas9 method. During the last years, Dr. Gruenert's group focused their attention on gene editing approaches using induced pluripotent stem cells (iPSCs) in order to develop novel therapeutic strategies for inherited diseases. He was the recipient of many CFRI Research Awards over the years. Tragically, Dr. Gruenert passed away unexpectedly in 2016. He is greatly missed. His groundbreaking and inspired research has led to new breakthroughs in the field of cystic fibrosis.

Remembering Isabel Stenzel Byrnes

Isabel Stenzel Byrnes, LCSW, MPH, a remarkable woman of grace, wisdom, strength, and compassion, passed away on July 12, 2023. Isa represents the heart and soul of CFRI. She lived with cystic fibrosis for 51 years, receiving a double lung transplant in 2004. Isa was part of our organizational family for nearly 30 years. In 2007, Isa and her late twin sister Anabel published the memoir, "The Power of Two." The sisters served as international patient advocates in their mother's country, Japan, which led to the creation of a documentary film of the same title. Isabel lectured around the U.S. on topics such as living well with illness, end-of-life issues and organ donation. Until very recently, she worked as a bereavement social worker at Mission Hospice. Isa was a beloved and active member of CFRI's Conference Committee and Diversity and Inclusion Advisory Committee. She facilitated CFRI's monthly grief support group for those who lost a loved one to CF, and was a forever honorary member of the Retreat Committee. Isa was a force of nature, a brilliant sage, the embodiment of generosity of heart and spirit. She is deeply missed.



Presentation Abstracts

*denotes CFRI-funded researchers

Role of CFTR Arginine-933 in FDA-Approved Drug Potentiation*

Friday, July 28, 9:15 am

Stephen Aller, PhD

University of Alabama, Birmingham, AL

Cystic fibrosis (CF) is caused by protein defects in the CF transmembrane conductance regulator (CFTR). CFTR is a difficult protein to study because: 1) it resides in the cell membrane; 2) folding is inefficient compared to other related membrane proteins; 3) many mutations that cause disease do not fold into functional protein, and 4) it is difficult to obtain sufficient monodisperse protein for biochemical or structural studies. Rare mutations at position 933 (R933) are known in the CF population but have not been comprehensively characterized by rigorous scientific methodology.

Breakthroughs in cryo-electron microscopy (cryo-EM) have allowed researchers to visualize high-quality structures with limited quantities of purified CFTR in unprecedented detail. Noteworthy are the 2019 structures by Liu et al of wild type CFTR describing the binding location of two different potentiator drugs - with one being the FDA-approved life extending Ivacaftor (VX-770). Potentiator drugs increase the open probability of the channel pore of CFTR. Interesting findings of this work include: 1) a channel pore that is not fully open despite the presence of potentiators, 2) the two different potentiators bind in the same pocket with highly similar orientations, 3) R933 is close to the pore and appears to directly interact with VX-770 and 4) no binding of VX-770 to R933A was detected. Anyone with knowledge of this literature might conclude that Ivacaftor would not be useful in treating 933 site-specific CF disease. My laboratory initiated in-depth studies of R933 mutations and their effects on CFTR folding phenotypes, electrophysiological characteristics, VX-770 efficacy, and possible shifts in VX-770 binding compared to wild type CFTR. We constructed and tested nine biochemically disparate mutations of R933. All nine mutations formed folded "band C" protein to various degrees and several were detected at levels considerably greater than wild type CFTR. Critically, VX-770 strongly enhanced chloride currents of all nine R933 mutations examined, including R933A which contrasts the 2019 findings. Cryo-EM studies are underway to determine if VX-770 binds to R933 mutants at the originally proposed site, or if a new site can be discerned. Importantly, these results suggest that VX-770 may offer therapeutic benefit in CF cases in which mutations occur at position 933.

Nanotechnology Toolkits for Cystic Fibrosis Gene Therapies*

Friday, July 28, 9:55 am

Steven J. Jonas, MD, PhD

University of California, Los Angeles, CA

Robust and reliable genome-editing tools are paving the way for innovative gene therapies that promise definitive cures for monogenetic disorders like cystic fibrosis (CF). Previous efforts to establish gene therapeutic strategies for CF have so far been met by significant challenges. For example, effective targeting and delivery of gene editing reagents to long-lived and self-renewing airway basal stem cell (ABSC) populations for gene correction in the CF lung has been especially difficult because of their protected location within the respiratory epithelium. This presentation highlights our multidisciplinary team's approach to overcome these obstacles through the design and testing of nanoparticle platforms configured to: i) expose access to ABSCs and ii) package and transport CRISPR/Cas9-based gene-editing cargoes for CFTR correction. We apply ABSC-derived air liquid interface (ALI) culture models that mimic the epithelial structure and organization of the human airway to test the delivery capabilities of the nanocarriers. The suite of tools and methods developed here serve as building blocks for establishing CF gene therapy solutions that are poised to accelerate progress toward our collective goal to find a permanent cure for CF on the steepest gradient possible.

Presentation Abstracts

Development of Bacteriophage Therapy for Antimicrobial-Resistant Infections in Cystic Fibrosis*

Friday, July 28, 10:50 am

Paul Bollyky, PhD

Stanford University, Palo Alto, CA

Bacteriophage therapy is a tantalizing therapeutic option for bacterial infections but is stymied by the narrow host range of individual phages. Combining multiple phages (phage cocktails) is attractive, but the fundamental principles governing phage-phage interactions are unclear. We have pioneered a novel approach to designing effective, broad-spectrum phage cocktails that relies on combining phages with different, complimentary receptors. By screening for bacteria with complete loss of phage susceptibility, we find that receptor usage is a major determinant of phage host range. Further, we identify what we term “complementarity groups” of phages sharing the same receptors.

Combining phages from multiple groups produces cocktails with broad host range. Moreover, targeting orthogonal receptor pathways simultaneously suppresses bacterial regrowth and preempts the emergence of phage resistance. Particular antibiotic classes have characteristic interactions with individual phage complementarity groups, making it possible to also predict synergistic phage-antibiotic combinations. Using this strategy, we generate phage/antibiotic cocktails effective against 95% of 174 *Pseudomonas aeruginosa* clinical isolates, including biofilm cultures. We likewise identify *Staphylococcus aureus* phage complementarity groups and cocktails, highlighting the broad relevance of this approach. Phage complementarity grouping provides a blueprint for identifying phage receptors, generating effective phage/antibiotic cocktails, and potentially revolutionizing the treatment of multidrug-resistant bacterial infections.

Airway Stem Cell Transplantation into the Sinuses Using Fibrinogen Scaffold*

Friday, July 28, 12:30 pm

Sriram Vaidyanathan, PhD

Nationwide Children's Hospital, Columbus, OH

Gene therapy approaches that restore CFTR function have the potential to treat all CF patients. However, delivery of the CFTR gene in vivo using viral and non-viral strategies has been unsuccessful. Airway stem cell therapies have been proposed as an alternative. However, cell therapies have been limited by concerns that conditioning regimens needed to make space for exogenous stem cells in the lung epithelium may be life threatening. Recognizing this, we propose to first transplant gene corrected autologous airway stem cells into the sinuses to treat CF sinus disease. We began by developing methods to genome edit upper airway basal stem cells (UABCs) from the nose and sinuses at high efficiencies. We recently reported the use CRISPR/Cas9 and adeno-associated viruses (AAV) to insert the full-length CFTR cDNA in the endogenous CFTR locus in airway basal stem cells. Epithelial sheets derived from corrected basal stem cells restore CFTR function to levels seen in non-CF controls. The transplantation of the corrected airway stem cells is the next technical hurdle that needs to be resolved for the clinical translation of this approach.

In preliminary in vivo experiments, we discovered that the delivery of cells in saline into the nasal cavity of mice resulted in their expulsion. Therefore, we attempted to identify biomaterial scaffolds that will adhere to the tissue and facilitate the engraftment of the gene corrected airway stem cells. We evaluated the ability of several materials including type I collagen, laminin foam-gel, fibrinogen, alginate, hyaluronan and dextran to support the survival and proliferation of UABCs. We used basement membrane extract from mouse tumors (Matrigel™) as controls. Among the materials tested, UABCs seeded in laminin and fibrinogen showed 5 ± 1 and 9 ± 6 fold expansion respectively compared to 4 ± 4 fold expansion in Matrigel. In addition, $>90\%$ of the UABCs cultured in laminin and fibrinogen gels maintained the expression of cytokeratin 5 after 4 days in culture. We then evaluated the ability of these materials

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Presentation Abstracts

Continued from page 27

to facilitate the transplantation of UABCs from mice that endogenously express firefly luciferase into immunocompromised NOD scid gamma (NSGTM)mice. In preliminary studies, the upper airways of NSG mice were injured using 2% povidone iodine and transplanted with UABCs using human fibrinogen and recombinant laminin. These mice exhibited stable bioluminescence for >60 days. We then attempted to transplant human UABCs into NSG mice using fibrinogen gels. Human UABCs were genome edited using CRISPR/Cas9 and AAV to express NanolucTM and GFP. UABCs expressing GFP were enriched using flow cytometry to >80% purity and transplanted into mice. Preliminary experiments show that the transplanted human UABCs produce a stable bioluminescent signal for ~120 days. Studies to evaluate the transplantation of UABCs from CF patients that have been genome edited showed engraftment of edited airway cells for over 60 days.

Tolerance to Cell Permeable Antibiotics: Intracellular Adaptations of *Pseudomonas aeruginosa**

Friday, July 28, 1:25 pm

Naren Kumar, B.Tech, PhD

University of California, Berkeley, CA

Persistent *P. aeruginosa* infections resistant or non-responsive to antibiotic treatment are a major concern for individuals with cystic fibrosis (CF). Recently we reported on a novel population that persists inside epithelial cells and tolerates treatment with high-dose antibiotics (100 X MIC). To better understand the intracellular location and bacterial factors enabling the intracellular resistance of *P. aeruginosa*, we utilized live time-lapse imaging and molecular methods to determine genes and proteins expressed by bacteria during intracellular infection of epithelial cells. Bacteria surviving high-dose antibiotic treatment were found to localize to Lamp3 labeled compartments within normal and CF cells in-vitro suggesting a vacuolar niche for surviving bacteria. Transcriptomic analysis of bacterial genes expressed by surviving bacteria identified the putative polyamine transporter *potB* (PA3608) as upregulated in bacteria within CF cells but not normal cells (3-fold increase). This suggests the involvement of the uncharacterized operon *potABCD* (homologous to the putrescine specific polyamine transporter *spuDEFGH* in *P. aeruginosa*) in the persistence of *P. aeruginosa* in CF cells. Thus, single gene transposon mutants in the *potABCD* operon were utilized to determine its role in in-vitro and intracellular susceptibility to ofloxacin.

Results showed that all mutants in components of the transporter (*Tn::potB* [PA3608], *Tn::potC* [PA3609], *Tn::potD* [PA3610]) became more susceptible to ofloxacin treatment in-vitro (0.25 - 0.50 vs 4 µg/mL). Interestingly, unlike *spuDEFGH* operon mutants that are capable of inducing biofilm formation, mutants in *potABCD* lost the capability to induce biofilm formation upon putrescine supplementation. In normal epithelial cells, intracellular survival was uniformly reduced for all mutants after ofloxacin treatment (1 µg/mL) [3-log reduction]. However, in CF cells only mutations *Tn::potD* and *Tn::potB* increased intracellular susceptibility to ofloxacin [2-log reduction]. In normal cells and WT bacteria, low biofilm (*cdrA*) and high T3SS (*exoS*) expression tend to be more susceptible to antibiotics. Thus, we hypothesized that the increase in susceptibility for the *potABCD* mutants will correlate with that expression profile. Surprisingly none of the mutants differed in the intracellular expression of the T3SS (~5% cells containing GFP+ bacteria) and only *Tn::potC* had an increased number of cells with *cdrA* reporting bacteria (~15%) relative to WT (5%). Here we report a novel mechanism wherein intracellular *P. aeruginosa* utilizes the putative polyamine transporter *potABCD* to alter its resistance to ofloxacin in CF cells. It remains to be determined if the mechanism of tolerance involves sensing and/or transport of host polyamines by intracellular *P. aeruginosa*.

Presentation Abstracts

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

Friday, July 28, 3:20 pm

Carlos Milla, MD; Nam Soo Joo, PhD
Stanford University, Palo Alto, CA

Mucociliary clearance (MCC) is a vital respiratory innate defense mechanism that is defective in CF. Impaired MCC in people with CF contributes to mucus obstruction, airway inflammation and infection that cause airway damage and lung function decline. We discovered that combining a low dose cholinergic agonist with a β -adrenergic agonist acts synergistically to markedly increase MCC velocity (MCCV) in ex vivo tracheal preparations from both ferrets and newborn piglets. Importantly, tracheae from transgenic CF ferrets also respond, increasing MCCV to a value ~55% of that seen in WT animals. The combined agonists do not induce airway narrowing (Joo et al., *Sci Rep*, 2021, 11, 18828). Our data indicate that synergistic increases in MCC are driven by synergistic increases in submucosal gland secretion, suppression of ENaC mediated absorption, and increased bicarbonate secretion and ciliary beat frequency. To better understand and extend these findings, experiments are currently underway to measure airway surface liquid heights in ex vivo animal tracheas, and MCCV in an in vivo sheep model of CF.

Pathways Balancing Basal Mucin and CFTR-Mediated Fluid Secretion in the Human Distal Airway*

Friday, July 28, 4:15 pm

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

Background: Mucus concentration is a key parameter that regulates mucus biophysical properties and is tightly regulated by luminal mucin secretion and mucus hydration. While small airways (< 2 mm in diameter) constitute the earliest, most affected region in CF, how mucus concentrations are regulated in the small airway region remains unknown. Our prior studies localized both MUC5B secretion and CFTR-mediated ion transport activities to small airway secretory cells. Here, we sought to identify pathways balancing basal mucin and CFTR-mediated fluid secretion in small airway secretory cells.

Methods: Single cell RNA-seq (scRNA-seq) was performed on matched large and small airway epithelial (LAE and SAE, respectively) cultures (N=5) to transcriptionally characterize region-specific airway epithelial cell types. A pathway enriched in small airway secretory cells was pharmacologically inhibited to test whether it regulated MUC5B secretion and CFTR function in SAE cells. A lentiviral vector that selectively transduced the wild-type (WT) CFTR gene into CF SAE secretory cells under the control of the rat club cell specific protein (rCCSP) promoter was used to test the competency of small airway secretory cells for CFTR function restoration.

Results: A total of 36,966 cells (LAE 57%, SAE 43%) were analyzed by scRNA-seq. scRNA-seq characterized small airway secretory cells by: 1) high levels of small airway marker gene expression (SFTPB, SCGB3A2); 2) high CFTR and MUC5B expression; 3) high expression of the IL-1 receptor gene (IL1R1); and 4) upregulation of downstream genes of IL-1 signaling pathways. To test whether MUC5B and CFTR-mediated fluid secretion is regulated via endogenous IL-1 signaling in small airway secretory cells, ion transport function and MUC5B secretion rates were measured in SAE cultures where IL-1 signaling was inhibited by IL-1 receptor antagonist (IL1Ra) administration. Baseline expression of IL-1 downstream genes was suppressed in IL1Ra-treated SAE cells. Notably, baseline MUC5B secretion rates and CFTR-mediated Cl⁻ secretion were also reduced in IL1Ra-treated SAE cells. Utilizing the lentiviral vector, the WT CFTR gene was transduced via the rCCSP promoter in CF SAE cells. Whole mount immunohistochemistry revealed colocalization of CCSP and CFTR protein in non-ciliated cells in rCCSP-CFTR-transduced CF SAE cultures. rCCSP-CFTR-transduced CF SAE cultures exhibited WT CFTR protein expression with restored CFTR-mediated Cl⁻ secretion in response to forskolin.

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Conclusions: Small airway secretory cells regulate basal mucin and CFTR-mediated fluid secretion at least partially by endogenous IL-1 pathways in human distal airways. These small airway secretory cells are a therapeutic target for molecular therapy for CFTR function restoration.

A Journey Through Rare: Because EVERYONE Deserves More Tomorrows

Friday, July 28, 6:00 pm

Rachel Alder
Salt Lake City, UT

A journey through rare together. Rae will share her timeline of navigating the world of rare leading up to her CF diagnosis just this year at the age of 26. Rae will include a timeline of symptomatology, along with pivotal life experiences subsequently leading her to the world of patient advocacy for everyone facing cystic fibrosis.

Phage Therapy and Cystic Fibrosis

Saturday, July 29, 9:00 am

Saima Aslam, MD, MS
University of California, San Diego, CA

Dr. Aslam will use a case-based approach to discuss the basic concepts of phage therapy, review current published literature on the topic and discuss ongoing research, including clinical trials in this field.

Bacteriophages (phages) are ubiquitous in the environment and are present within the human microbiome as well. Phage therapy constitutes the usage of lytic-only phages targeted to specific pathogens with clinical intent. Several successful cases of phage therapy in the setting of cystic fibrosis will be discussed including use in multidrug resistant *Pseudomonas aeruginosa*, *Burkholderia cenocepacia* complex and *Mycobacterium abscessus* infections. The phage therapy clinical experience at UC San Diego will be reviewed; thus far we have a success rate of almost 80% in antibiotic recalcitrant infections treated at our center. A variety of different factors play a role in the clinical effectiveness of phage therapy including the type of phage used, multiplicity of infection, synergy with other phages and/ or antibiotics, concentration of phage used, mode of administration, stability of the clinical preparation, development of serum neutralization, and type of infection treated, among others. In general, phage has been found safe for clinical use. Ongoing research in the area of cystic fibrosis and multidrug resistant pathogens will be discussed.

All Hands On Deck To Cure Cystic Fibrosis

Saturday, July 29, 10:05 am

Matthew Porteus, MD, PhD
Stanford University, Palo Alto, CA

In the original formulation of genome editing it meant that one could change the sequence of the genome just as one could change a document on your computer. Recently genome editing has been most broadly used to make gene knockouts, rather than directly fix genetic variants that cause disease. Yet, direct correction of mutations is ultimately the most powerful approach to transforming medicine for those with genetic diseases. The most versatile approach to gene correction is using genome editing by homology directed repair (HDR). Using HDR we can correct a wide variety of disease-causing mutations using a single strategy.

We have applied this approach to developing a one-size fits all approach for the thousands of mutations that cause cystic fibrosis. The HDR approach works best in engineering cells outside the body (“ex vivo”). We have used genome editing to correct cystic fibrosis causing mutations in basal cells (stem cell of the airway) and now demonstrated that those cells can repopulate the epithelium of the sinus in mice. I will discuss our next steps needed to obtain FDA clearance to test the approach to treat serious sinus disease in cystic fibrosis patients.

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Understanding and Managing Pain in CF: A Biopsychosocial Approach

Saturday, July 29, 11:15 am

Amanda S. Bruce, PhD and Deborah Friedman, PhD

University of Kansas Medical Center, Kansas City, KS; Massachusetts General Hospital CF Center, Boston, MA

Pain is a common, significant symptom for people with cystic fibrosis (CF) at all stages of disease severity. Pain affects up to 75% of children and 89% of adults with CF. It can have a negative impact on mood, quality of life, and overall physical functioning, and can interfere with an individual's ability to engage in treatments for CF, school, work, and daily life activities. However, this topic has been understudied in CF.

Treatment for pain ideally involves a multi-component approach that may involve both medication and non-medication interventions. A specific non-medication approach to pain management, called cognitive-behavioral therapy (CBT), has been shown to be effective for reducing pain and its interference in daily life with a low risk of side effects in other chronic diseases. CBT is also effective in treating depression, anxiety, and sleep problems that often co-occur with pain. However, it can be difficult for people with CF to access CBT to manage pain, and standard protocols need to be tailored to meet the unique needs of people with CF.

We are currently conducting a research study supported by the CF Foundation to develop a brief, telehealth, CF-specific mind-body program for pain management. This program will draw from well-established CBT and acceptance-based approaches. We will integrate these empirically supported techniques with CF-specific content drawn from feedback from community stakeholders. Our multi-center research team has demonstrated feasibility, acceptability, and effectiveness of a similar CF-specific 8-session CBT program for the prevention and treatment of depression and anxiety (CF-CBT). CF care team members at centers across the U.S. and Canada are currently being trained to provide this program to patients as part of routine CF care. Our pain management study will follow a similar model by eliciting input from the CF community and CF care team members through individual interviews and focus groups to develop a pain management program that addresses the specific needs of people with CF. In our presentation, we plan to discuss our findings from stakeholder interviews about the experience of living with both CF and chronic pain, as well as describe evidence-based mind-body approaches to pain management.

From Defining Health Disparities to Improving Health Equity in Cystic Fibrosis

Saturday, July 29, 1:15 pm

Susanna A. McColley, MD, FAAP, ATSF

Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, IL

The term "health disparity" is used to describe a difference in health, in which a specific characteristic leads to better or worse health in a population. "Health inequity" is a difference in health that results from unfairness and injustice. Health disparities have been reported in cystic fibrosis for decades. For example, people with CF from lower socioeconomic status (those with fewer financial resources) have worse health outcomes across continents and countries with different health care, insurance, and CF care systems. There are also worse health outcomes in people with CF from minoritized racial and ethnic groups, even when taking socioeconomic status into account. Both are also true in many other common and rare diseases. In CF, scientific and public health advances have made a major impact on the health of the CF population but have widened disparities.

In this presentation, we will discuss how CF clinical trial enrollment, newborn screening, and global availability create inequities. We will also discuss how policies and practice must advance so that every person with CF has a fair and just opportunity to attain their highest level of health.

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Panel: Paths to Parenthood with CF

Saturday, July 29, 2:45 pm

Lucy Barnes; Matthew DeFina; Carl Robinson

Ashland, OR; Napa, CA; San Jose, CA

Moderated by Mary Helmers, RN

Lucile Packard Children's Hospital Stanford, Palo Alto, CA

Improved treatments for cystic fibrosis – most notably CFTR modulators – and resulting increased life expectancy has translated to larger numbers of individuals with CF becoming parents. Speakers on this engaging panel will share their individual paths to parenthood, including adoption, in vitro fertilization (IVF), and pregnancy/childbirth. Moderated by Mary Helmers, who has counseled many of her patients with CF about available options as they weigh the decision to become parents, the discussion will explore panelists' range of experiences related to reproductive health discussions with their CF care providers; their partners' key roles; finding balance with parenting, working and managing CF; the impact of pregnancy and/or parenthood upon health maintenance; and addressing psychosocial care needs.

Advances in mRNA Therapy: New Applications for Cystic Fibrosis

Saturday, July 29, 3:30 pm

Deepika Polineni, MD, MPH

Washington University School of Medicine, St. Louis, MO

Cystic fibrosis is caused by mutations in the cystic fibrosis transmembrane conductance regulator gene (CFTR) leading to defective CFTR protein. It is estimated that up to 10% of people with cystic fibrosis (CF) in the United States are unable to benefit from currently FDA approved CFTR modulator therapies due to their CFTR mutations (i.e., genotype) or a history of side effects to such treatments. Research is underway for alternative strategies to improve the health of people with CF who do not benefit from CFTR modulators. Messenger ribonucleic acid (mRNA) therapy is one such treatment option that is under investigation for people with CF and has often recently been included under the term “genetic therapies” for CF. Importantly, mRNA therapy is distinct from gene therapy and gene editing. mRNA is a type of ribonucleic acid that is present in human cells and represents one step in the process of the DNA genetic code becoming translated into a functional protein. Using mRNA replacement therapy as a treatment in CF involves the careful delivery of mRNA coding for CFTR into airway cells to use the cells' own machinery to create normal CFTR protein in the lungs. The success of this depends on many factors including the stable maintenance and delivery of the therapy.

Recently, mRNA was utilized successfully in development of mRNA-based SARS CoV-2 vaccines for COVID-19 during the pandemic. Lessons learned from these vaccines and the pandemic will continue to inform investigations of mRNA in therapeutic development and use. mRNA therapy is now under early phases of study for safety and tolerability in people with CF who cannot benefit from CFTR modulators based on their CFTR mutations. mRNA could also have broader future applications for people with CF independent of their CFTR mutations. In summary, mRNA therapy in CF is a new treatment under investigation that could have potential to improve lung disease for people with CF irrespective of their CFTR genotype. This presentation will provide a review of mRNA as a novel therapeutic option.

Embracing the Future: Aging with CF

Sunday, July 30, 9:15 am

Ahmet Uluer, DO, MPH

Boston Children's Hospital/ Brigham & Women's Hospital, Boston, MA

Cystic fibrosis (CF) is a multisystem disorder primarily affecting the respiratory and digestive systems. Advancements in treatment, including highly effective modulators, have significantly improved the life expectancy and quality of life for individuals with CF. The impact of aging is becoming more important as awareness for complications associated with CF is growing. It is crucial to screen for these complications

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and recognize symptoms early, including cancer involving the gastrointestinal (GI) tract, complications associated with diabetes, hypertension, bone disease, hearing loss, kidney disease, cardiovascular disease, among others.

The incidence of cancer involving the GI tract occurs at higher rates for people with CF than the general population, particularly those receiving organ transplant. Regular screening and surveillance are essential to detect and not only manage but even prevent malignancies. Individuals with CF are at an increased risk of developing diabetes and over 35% have this listed as a diagnosis. Monitoring for elevated blood pressure also important, especially for those with diabetes. Furthermore, people with CF are also at risk of hearing loss and kidney disease associated with life-saving treatments. Monitoring and screening measures are important to prevent and manage both kidney disease and hearing loss. If necessary, interventions involving hearing aids and cochlear implant can impact quality of life. Cardiovascular disease is also emerging as a concern among adults with CF. Regular cardiovascular screening, including monitoring of lipid profiles and assessment of cardiac function, is necessary to identify and manage cardiovascular complications promptly. Early identification and aggressive management are essential to mitigate their impact on overall health and well-being.

In conclusion, as the adult CF population continues to grow, it is imperative to recognize the complications associated with aging in individuals with CF. Screening, early treatment, and understanding of effective management strategies are essential to improve the quality of life for adults living with CF.

CF and Colon Cancer: My Lived Experience

Sunday, July 30, 9:15 am

Anna Payne
Langhorne, PA

Anna Payne is a 36-year-old cystic fibrosis and colon cancer patient. She is also the founder of the Bucks County CF Alliance, a non-profit organization. In her talk, she describes her experience with CF and living with stage-4 colon cancer. She was diagnosed at the age of 34, well before the recommended screening age of 40. Those with cystic fibrosis have 5 to 10 times the rate of colon cancer as the general public: those post transplant have over 25 times the risk. After her diagnosis, Anna immediately got to work, seeking to educate as many people as possible about the elevated risk that CF patients and carriers have of developing colon cancer. She will detail her story, from diagnosis until today; what she has learned and what we can do to help make sure this doesn't happen to others.

Presenting alongside Dr. Ahmet Uluer, Anna will share the personal side of this two-part medical story: the real-life impact that colon cancer has on people and what it looks like living with two diseases.

Advances in Stem Cell Research for the Treatment of CF

Sunday, July 30, 10:15 am

Brigitte Gomperts, MD
University of California, Los Angeles, CA

This is a very exciting time in CF research. The first clinical trial of nebulized CFTR mRNA airway delivery has been completed and several more clinical trials are in the pipeline. While we await the results of these trials, research is moving forward with even more advanced gene therapy approaches. But central to understanding how gene addition or gene editing will work for CF is knowing which cells in the airway should be targeted. We will share our recent findings using single cell RNA sequencing of the multitude of different cell types and subtypes in the human airway and their function. We will also discuss how these cell subtypes change in CF. We will focus in on which cell types express CFTR and examine the different airway stem cells. Airway stem cells are of particular interest because gene editing of these cells could potentially lead to long-lived correction of CFTR.

We will then review the current approaches to replace, repair or restore the CFTR gene in the airway, which include mRNA delivery, gene delivery, gene editing, and cell therapy approaches. Cell therapy

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approaches are in their infancy, and we will explore the different kinds of stem cells that could potentially be used and the pros and cons of each stem cell type.

There are several barriers to airway delivery of these gene therapeutic approaches which are even more challenging in CF, such as thick, tenacious mucus and inflammation. There are also barriers to systemic gene delivery and gene editing in the body. Cell therapy approaches have their own delivery issues and the additional hurdle of engraftment in the airway. We will discuss each of these barriers and potential ways to overcome these obstacles with advances in delivery systems. Overall, we will show major progress in the field of gene therapy for CF that is being made through cutting edge research in this area and provides hope that this could provide a therapeutic approach for all CF patients.

Turning Struggles Into Strengths

Sunday, July 30, 11:20 am

Alanah Rosenbloom, MSW

San Jose, CA

Navigating a life with CF is tough... to say the least! Having CF can feel weird, gross, and unpredictable, especially when all you yearn for is to be normal and in control. It's taken me decades to gain some perspective on the struggle that is living with CF, and while I don't necessarily feel normal or in control, I do feel incredibly empowered by the experience thus far. After living with CF for 37 years, I'm ready to share some of my stories.

Join me as I talk about:

- The funny, the bad, and the ugly
- Befriending others with CF
- Using CF to be of service to others

I'm excited and honored to share my experience living with this disease with people like you – who really get it. Whether you have CF or not, I hope this talk generates hope that what once brought shame can one day become cherished.

Support & Discussion Group Guidelines

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 are offering the following 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 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 licensed and practicing professional counselors. They are required by law to report incidences of child, elder or spousal abuse.
- Respect the members of your support group, including their situations, emotions and perspective. 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.

Special Thank you

CFRI expresses its sincere gratitude to the following individuals who played a key role in bringing this conference to fruition.

CFRI Professional and Volunteer of the Year Awards Panel

Francine Bion
Colleen Dunn, MS, RT, CCRD
Oscar Flamenco, CPA
Marina Gonzales
Danielle Mandella
John Mark, MD
Carole Nakamura, RN
Dennis Nielson, MD, PhD
Anna Payne
Yelizaveta Sher, MD, FACLP

CFRI Conference Committee

Francine Bion
Sabine Brants, MA
Isa Stenzel Byrnes, LCSW, MPH
Mary Convento
Barbara Curry
Leeya Kannankunni
Jane Mitchell, MSW
Stacie Reveles
Ann Robinson
Siri Vaeth, MSW

Award Celebration Special Guests



Rae Alder

This year Rae overcame racial bias, health disparities, and rapid health deterioration to finally receive the correct diagnosis of cystic fibrosis at the age of 26. As shared in her Speaker Bio, Rae has crafted a life's work centered in the art of advocacy and authenticity. In addition to her advocacy work, Rae is a talented vocalist, public speaker, and is a National Champion in After Dinner Speaking for her university. In 2019, Rae lost a wager with a friend, resulting in her entering the Miss North Ogden scholarship program. She won and advanced to the state competition. Rae firmly believes in the power and importance of sharing our own authentic journeys. These are her greatest passions.



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.



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.



In-Person 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.

An introduction to some frequently used cystic fibrosis related terms:

absorption — the process of transporting nutrients from the intestine into the bloodstream for use by the rest of the body.

ADEK/ABDEK — vitamins A, D, E, and K are fat-soluble (vs. water-soluble) vitamins. Fat-soluble vitamins are important for general good health, daily repair of the body cells, and functioning of the organs.

aerosol — a mist for inhalation, usually containing medicine.

ACT — **airway clearance technique**; for example PEP (positive expiratory pressure), Acapella®, Aerobika® Flutter®, chest percussion, high frequency oscillating vest.

aspergillus — a fungus that is often found in the airways of people with cystic fibrosis (CF). People can develop an allergic reaction to *aspergillus*, called Allergic Bronchopulmonary Aspergillosis (ABPA). ABPA affects approximately 2% to 11% of people with CF, causing inflammation in the lungs which can cause scarring or bronchiectasis.

autosomal recessive — a genetic trait or disorder that appears only when a person inherits a pair of chromosomes – one from each parent – each with the gene for the trait. CF is autosomal recessive.

BMI (body mass index) — the measure of body fat based on height and weight that applies to adult men and women.

bronchiectasis — a condition in which damage to the airways causes them to become stretched, widened, and scarred, and unable to clear mucus, thus impacting their ability to move oxygen in and out of the lungs.

Burkholderia cepacia complex — a type of bacteria that can occur in CF. There are five strains (genomovars), each one with different degrees of clinical impact. *B. cepacia* can be very contagious or lethal, depending on the strain.

carriers — people with a single gene for a genetic condition like CF. Carriers do not have the disease.

CBAVD — congenital bilateral absence of the vas deferens, which is very common in men with CF.

CFRD (cystic fibrosis-related diabetes) — neither type 1 nor type 2 diabetes, CFRD is another type of diabetes that occurs in approximately 35% of young adults with CF, and 43% of those with CF over 30 years old. As with all diabetes, the body is unable to move sugar from the blood into the cells for energy and may need to be treated with insulin.

CFTR (cystic fibrosis transmembrane conductance regulator) AKA the CF protein — this gene provides instructions for making a protein of the same name. The protein functions as a channel that transports chloride across certain cell walls.

CFTR Modulators — small molecules that target specific defects caused by mutations in the CFTR gene. They are classified into three main groups: Potentiators, Correctors and Production correctors.

chest physical therapy (CPT or PT) — an airway clearance technique that often includes postural drainage and percussion.

cilia — tiny hair-like projections in the nose, trachea and bronchi, which, through their coordinated movement, help move mucus and particles.

clinical trials — studies to evaluate the effectiveness and safety of medications or medical devices by monitoring their effects on large groups of people.

Glossary

clubbing — rounded, enlarged tips of the fingers and toes. In CF, clubbing is thought to be caused by a chronic shortage of oxygen in the blood.

digestive enzymes or enzymes — juices produced by the pancreas that break down the carbohydrate, fat and protein in food. Some people with CF have a lack of these juices and take enzyme capsules to aid in digestion.

DIOS (distal intestinal obstructive syndrome) — unique to individuals with cystic fibrosis, DIOS involves blockage of the intestines by thickened stool. Previously known as meconium ileus equivalent (MIE), this syndrome is relatively common, occurring in about 10% – 22% of individuals with CF.

endoscopic sinus surgery — surgery to enlarge the drainage pathways of the sinuses that is performed through the nostrils with small cameras, avoiding the need for external incisions.

exacerbation (pulmonary exacerbation) — a lung infection, or worsening pulmonary symptoms, including increased cough and sputum production and/or shortness of breath, accompanied by an acute decrease in lung function.

FEV1 (Forced Expiratory Volume in 1 second) — the maximal amount of air you can forcefully exhale in one second during spirometry or pulmonary function testing. It is reported as a percentage of normal (a comparable person without lung disease), based on your height, weight and race.

FVC (Forced Vital Capacity) — the total amount of air in the lungs, usually the first number on the report from a pulmonary function test. It is measured in liters or as a percentage of normal.

G-tube (J-tube, button) — a feeding tube placed through the abdominal wall into the stomach or intestine for supplemental nutrition.

GERD (gastric esophageal reflux disease) — a condition of increased acid concentration and an increased tendency for acid regurgitation from the stomach in the mouth and lungs of the patient.

gene — a sequence of DNA that codes for a protein, which is used for a particular function such as building tissues, organs or other substances in your body.

genotype — the genetic makeup of a cell (i.e. the specific allele of the individual cell), usually with reference to a specific character under consideration.

hemoglobin A1c (HbA1c) — a measure of average blood glucose levels over the recent weeks or months; over 6.5% is considered diabetic.

hemoptysis — coughing up blood, or bloody mucus from the lungs.

heterozygous — organisms with two different alleles, or versions, of a given gene.

homozygous — organisms with two copies of the same allele, or version, of a given gene.

hyperglycemia — higher than usual level of glucose in the blood.

hypoglycemia — literally meaning “low blood sugar,” hypoglycemia is a condition in which blood glucose levels are abnormally low.

IgE or IgG — a type of antibody level found in the blood that indicates exposure to certain allergens or an immune response.

Glossary

in vitro fertilization (IVF) — a process that involves monitoring and stimulating a woman’s ovulatory process, removing ova from her ovaries and fertilizing them with sperm in a culture medium before implantation.

malabsorption — poor uptake of nutrients from food. In CF, mucus may plug ducts of digestive organs and block the secretion of enzymes and hormones, leading to malabsorption.

meconium ileus — blockage of the intestines of a newborn with very thick meconium (the first newborn stool). It can be the earliest symptom of CF and occurs in 7% – 10% of people with CF.

MDI (metered dose inhaler) — also known as a “puffer,” it is used to deliver medication to open up the lungs or reduce inflammation.

methicillin resistant *Staphylococcus aureus* (MRSA) — a bacterial infection or colonization that is highly resistant to most antibiotics, and often treated with vancomycin.

microsurgical epididymal sperm aspiration (MESA) — surgical extraction of sperm to be used for in vitro fertilization.

modifier genes — genes that impact other gene outcomes. For example, if a person has the obesity gene and the CF gene, perhaps the person will be less likely to suffer from poor growth or weight maintenance if he/she has poor pancreatic function.

motility — refers to the forward movement of ingested nutrients through the GI tract.

mRNA therapy — treating CF by delivering mRNA to the airways that encodes CFTR; a potential therapy in clinical development to treat all CFTR mutations.

mucociliary clearance (MCC) — the mechanical elimination of fluid, bacteria and particulates from the respiratory tract.

mucolytics — medicines that thin mucus, making it easier to cough out the mucus. Examples include hypertonic saline.

mucus plugs — thick mucus in a duct or airway that can block the flow of secretions or air.

mutation — changes, or mutations, in the CFTR gene cause cystic fibrosis. Nearly 2,000 mutations have been identified, and have been divided into five classes, based on how the CFTR protein is affected.

nasal polyps — small growths of swollen mucus membrane that project into the nasal passages. They can be surgically removed.

nebulizer — a device used with an air compressor that turns liquid medication into a mist so that it can be inhaled directly into the lungs through a mask or mouthpiece.

Nonsense mutations — also known as “X” or “stop” mutations, this occurs in DNA when a sequence change leads to a stop codon rather than a codon specifying an amino acid, causing the production of CFTR protein to stop prematurely. Approximately 10% of people with CF have these mutations.

non-tuberculous mycobacterium (NTM) — species in the family of mycobacteria that may cause human disease, but do not cause tuberculosis (TB). The most common NTM’s cultured among those with CF are *M. avium*, and *M. abscessus*.

Glossary

oral glucose tolerance test (OGTT) — a blood test that measures the body’s ability to use a type of sugar called glucose which is the main source of energy for cells. An OGTT can be used to diagnose diabetes.

oxygen saturation — amount of oxygen carried by the hemoglobin in the blood. This is measured by a pulse oximeter (using infrared light on a finger) or by a blood gas test, where blood is drawn from the artery in the wrist.

pancreas — the long organ behind the stomach which secretes enzymes through ducts into the intestine to break down food. In CF, you can be pancreatic sufficient, whereby your pancreatic enzymes are secreted normally, or pancreatic insufficient, whereby your pancreatic enzymes are blocked by mucus and you need supplemental enzymes.

pathogen — a microbe or microorganism such as a virus, bacterium or fungus.

percussion — an airway clearance technique that involves clapping on the chest with a cupped hand, or vibrating the chest with another device, to loosen mucus in the lungs.

PERT — pancreatic enzyme replacement therapy. Almost 90% of people with CF need to take replacement enzymes prior to eating to aid with digestion and nutrient absorption.

phenotype — in genetics, this is the term used to describe a patient’s observable characteristics or traits.

postural drainage — an airway clearance technique that involves lying in various positions to drain mucus from the lungs.

PPI (proton pump inhibitor) — a type of medication that suppresses acid production in the stomach.

***Pseudomonas aeruginosa* (PA)** — a type of bacteria that often lives in the lungs of people with CF and causes lung infections.

PFT (pulmonary function test) — a group of tests that measure how well a person’s lungs are working and can help determine disease progression by tracking changes in lung function over time. The current recommendation is that people with cystic fibrosis have PFTs done at least four times per year.

rectal prolapse — protrusion of the rectum, which may occur in children with CF because of digestion problems. This condition can lead to a CF diagnosis.

spirometer — a device that measures air flow and lung volume.

sputum — mucus from the lungs; phlegm.

sputum culture — a microbiology test to separate and identify bacteria or fungi infecting the lungs.

***Staphylococcus aureus* (staph)** — a type of gram-positive bacteria that can cause numerous types of infections. In CF, *staph* often causes lung infections.

Stenotrophomonas maltophilia — a multi-drug resistant gram-negative bacteria that causes lung infections.

surgical navigation system — a computer-assisted process that helps surgeons to identify critical landmarks and enhance safety during surgery, e.g., sinus surgery.

throat culture or “gag” sputum — a test to identify a bacterial or fungal infection in the lungs; used when the patient cannot cough up sputum.

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 39th Annual Golf Tournament Benefitting CFRI at the beautiful Cinnabar Hills Golf Club in San Jose on Monday, August 14, 2023.
- CFRI's Gala, "A Breath of Fresh Air," will be held Saturday, October 14, 2023, in-person at the Hillsborough Racquet Club (Hillsborough, CA) as well as virtually. 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 24-HOUR VIRTUAL DANCE PARTY — The second event was held February 24, 2023, with dozens of dancers from across the country logging in and dancing over a period of 24 hours. Seek pledges, dance and support CFRI's wellness programs.

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 \$120 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, 6:00 pm PT to 7:30 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 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 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 two CF social workers well versed in issues facing teenagers with CF. Parents need to give consent for their teenagers to attend.

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 Sonya Haggett, LCSW, adult with CF and lung transplant recipient.



CFRI Programs and Events

Zoom Into Now

Fourth Tuesday of every month, 5:00 pm to 6:15 pm PT. Monthly Mindfulness sessions for the CF community, led by Dr. Julie Desch. Each session combines mindfulness practices and meditation, which has been shown to reduce anxiety and depression.

CFRI's CFQoL Programs are generously supported by Chiesi USA, Viatrix, Genentech, Horizon Therapeutics, Gilead Sciences, Vertex Pharmaceuticals, Boomer Esiason Foundation, 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, 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, Viatrix, Gilead Sciences, Genentech, and Chiesi USA.



CFRI Programs and Events



CF Spring and Summer Retreats

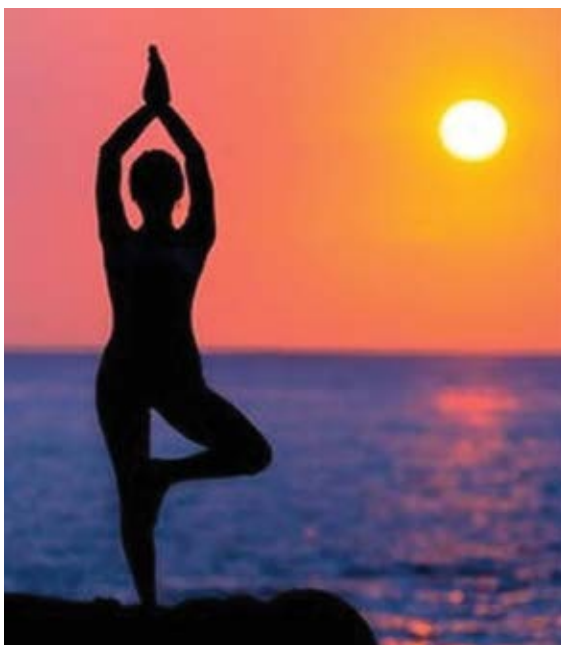
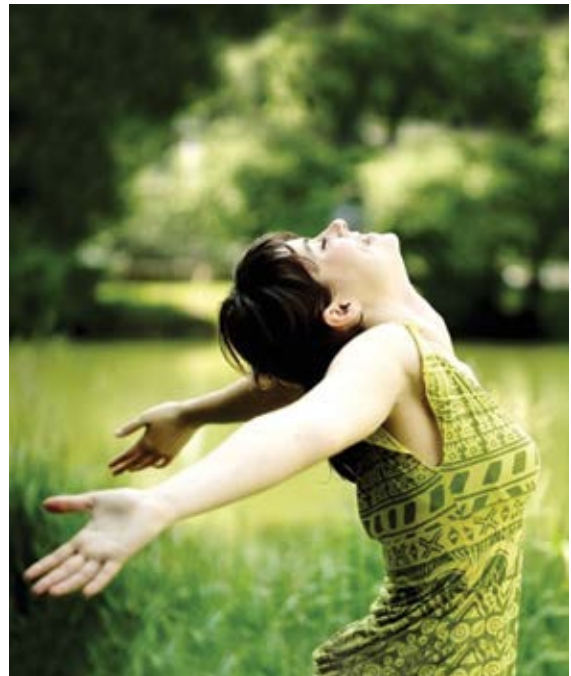
The annual CF Spring Retreat and CF Summer Retreat enhance education, positive coping skills, and social support for people who share common experiences with CF, and include educational presentations, exercise, arts and crafts, support groups, and much more. The 2023 Summer Retreat will be held at Vallombrosa Retreat Center in Menlo Park, CA from August 18 to 22. **Join us!**

Generously sponsored by AbbVie, Vertex Pharmaceuticals and Gilead Sciences.

Embrace Retreat for Mothers of Children and Adults with Cystic Fibrosis

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, Vertex Pharmaceuticals and Gilead Sciences.



CF Wellness Classes

CFRI's wellness classes for the CF community are held on alternating Thursdays and Saturdays. Classes are open to individuals with CF, as well as parents, spouses, partners and siblings of those with CF and provide opportunities to improve the physical and emotional health of those with CF and their family members. No experience is required, and all abilities and mobilities are welcome!

Generously sponsored by Viatrix, Vertex Pharmaceuticals, and private donors.

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, COVID-19, and more.

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



Purple Hair Challenge

Each May during CF Awareness Month, we challenge the community to dye your 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 and Chiesi USA

A Breath of Fresh Air Virtual Gala Event

On Saturday, October 14, 2023, join us in person or online for our annual gala and support the search for a CF cure. The in-person event will be held at the Hillsborough Racquet Club in Hillsborough, CA. In addition to inspiring stories, musical performances, and celebrity appearances, we will honor our 2023 CFRI Champion.

Generously sponsored to date by Vertex Pharmaceuticals, AbbVie, Chiesi USA, and Viatrix.

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





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.

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