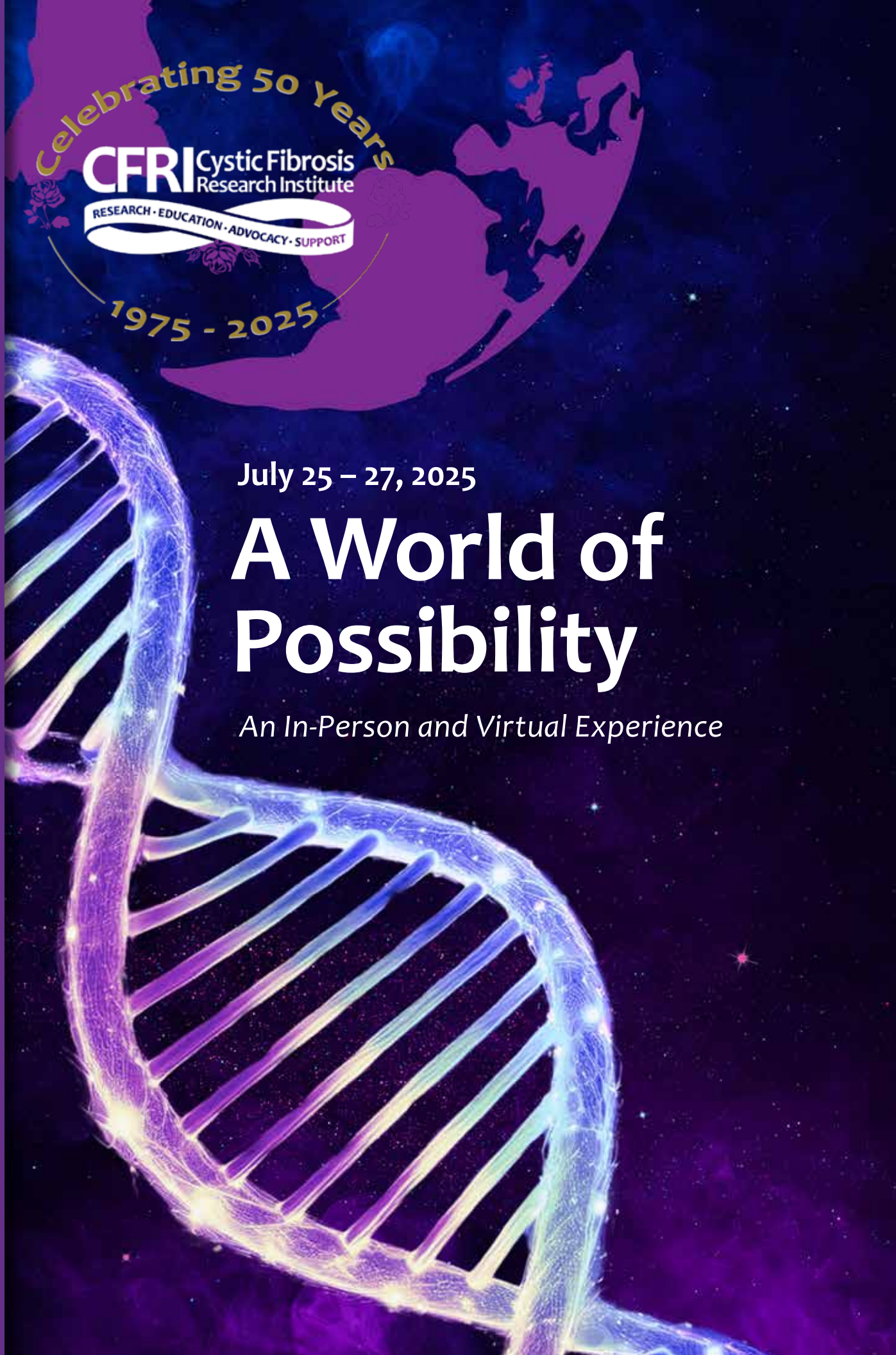
38th National Cystic Fibrosis Education Conference

Celebrating 50 Years

CFRI Cystic Fibrosis Research Institute

RESEARCH · EDUCATION · ADVOCACY · SUPPORT

1975 - 2025



July 25 – 27, 2025

A World of Possibility

An In-Person and Virtual Experience

Welcome

Dear Friends,

Welcome to CFRI's 38th National Cystic Fibrosis Education Conference, *A World of Possibility*. We are delighted to once again offer this year's conference as a hybrid event, that will offer the opportunity for all members of our community to come together in person and virtually, from across the nation and globe.

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 2025 conference provides the opportunity to hear from experts in the field of cystic fibrosis, addressing vital topics in research and clinical care. We are extremely grateful to all of our presenters, who generously share 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 2025 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 our exhibitors. They have been key partners in much of the progress that we celebrate.

CFRI remains steadfast in our 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. 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

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Conference Schedule — *A World of Possibility*

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

Friday, July 25, 2025

3:00 pm	**Registration Opens**
3:30 pm – 5:00 pm ***Pioneer*** ***Discovery*** ***Voyager***	**Support Groups** (In Person only) — *Adults with CF and Adults with CF Post-Transplant* — *Parents/Caregivers of Children with CF* — *Parents/Partners of Adults with CF*
5:30 pm – 7:00 pm ***Airstream***	**Reception and Welcome at the Airstream!**
7:00 pm – 7:45 pm ***Pioneer***	**Phages in Progress: Exploring Synergy, Resistance, and Therapeutic Potential** — Katrine Whiteson, PhD *(UC Irvine)*
7:45 pm – 8:30 pm ***Pioneer***	**Living Beyond Rare: My Journey with CF, Defying Limits with a Unique Perspective** — Jaelyn Cooper, MHA *(Inspirational Presentation)*

Saturday, July 26, 2025

Concurrent Sessions: Those listed in purple denote CFRI-funded research presentations.

7:45 am – 8:45 am ***Roger Restaurant***	**Breakfast**
8:45 am – 9:00 am ***Pioneer*** ***Orion***	**Welcome and Opening Remarks** — Siri Vaeth, *MSW, CFRI Executive Director* / Rohini McKee, *Emcee* — Devin Wakefield, *Research Track Emcee*
9:00 am – 9:45 am ***Pioneer***	**Addressing Pain in Cystic Fibrosis: Causes, Strategies, and Self-Advocacy** — Nicole Tovar, PT, DPT *(Endurance PT)*
Orion	**Elucidating the Ion Transport Functions of CFTR High Expresser *Cells* (CHEs) and Its Relevance to Intestinal Disease in CF** — Nadia Ameen, MD *(Yale University)*
9:55 am – 10:40 am ***Pioneer***	**Getting Older, Growing Bolder, Navigating Aging with CF** — Ahmet Uluer, DO, MPH *(Harvard Medical School /Brigham & Women's Hospital)*
Orion	**Optimization of Phage Therapy to Reduce *Pseudomonas*-Induced Inflammation in CF** — Benjamin Chan, PhD; Kaitlyn Kortright, PhD *(Yale University)*
10:55 am – 11:40 am ***Pioneer***	**Reproductive Health in People with CF** — Raksha Jain, MD, MSc *(University of Texas Southwestern)*
Orion	**Designing a CF Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cell-Derived Ionocytes** — Steven Jonas, MD, PhD; Ruby Sims, PhD *(University of California Los Angeles)*

11:50 am – 12:35 pm ***Pioneer***	**Increased Risk of GI and Other Cancers in People with CF** — Steven Freedman, MD, PhD *(Beth Israel Deaconess Medical Center / Harvard Medical School)*
Orion	**Characterization of Ionocyte Subtypes in Cystic Fibrosis Ferrets** — Feng Yuan, PhD *(University of Alabama at Birmingham)*
12:35 pm – 1:35 pm ***Infinity Foyer***	**Lunch**
1:45 pm – 2:30 pm ***Pioneer***	**Emerging Cardiovascular and Metabolic Risk Factors in the Era *of* Highly Effective CFTR Modulators** — Gregory Ratti, MD *(University of Texas Southwestern)*
Orion	**A Hybrid Gene Correction Strategy for Cystic Fibrosis** — Anais Amaya, PhD; Matthew Porteus, MD, PhD *(Stanford University)*
2:40 pm – 3:25 pm ***Pioneer***	**Living to Dream or Fighting to Breathe? Why Cystic Fibrosis Isn't Equal for All** — Meghan McGarry, MD, MAS *(University of Washington Seattle)*
Orion	**Corrected CFTR-F508del is Targeted for Endolysosomal Degradation** — Ron Kopito, PhD; Celeste Riepe, PhD *(Stanford University)*
3:40 pm – 4:25 pm ***Pioneer***	**Nucleic Acid-Based Therapies for CF: Progress and Challenges** — Joseph Pilewski, MD *(University of Pittsburgh)*
Orion	**Optimization of Activity and Improved Delivery of Bacteriophages Targeting *Burkholderia spp*** — Daria Van Tyne, PhD *(University of Pittsburgh)*
4:30 pm – 6:00 pm ***Artemis***	***Innovations and Libations* Reception** (in-person only)
6:00 pm – 8:00 pm ***Ballroom***	**CFRI Awards Celebration & Dinner with Special Guests**
8:00 pm – 10:30 pm ***Ballroom***	**Dance Party with DJ** (in-person only)

Sunday, July 27, 2025

8:15 am – 9:15 am ***Roger Restaurant***	**Breakfast**
9:15 am – 9:30 am ***Pioneer***	**Welcome / CFRI Overview** — Siri Vaeth, *MSW, CFRI Executive Director*
9:30 am – 10:30 am ***Pioneer***	**Panel: Mental Health Impacts of CF Upon the Family** — Jacob Fraker, MSW; Sarah Modlin-Tucker, DO; Hema Patel; Gail Wright, RN; Moderated by Deb Menet, LCSW *(Stanford CF Center)*
10:30 am – 11:00 am	**Break** — Room Check-Out
11:00 am – 12:10 pm ***Pioneer***	**Guts, Glitter, Glory** — Dylan Mortimer, MFA Presentation, Followed by Interactive Art Workshop
12:10 pm – 12:15 pm ***Pioneer***	**Closing Remarks** — Siri Vaeth, *MSW, CFRI Executive Director*

Tips for Navigating the Virtual Conference

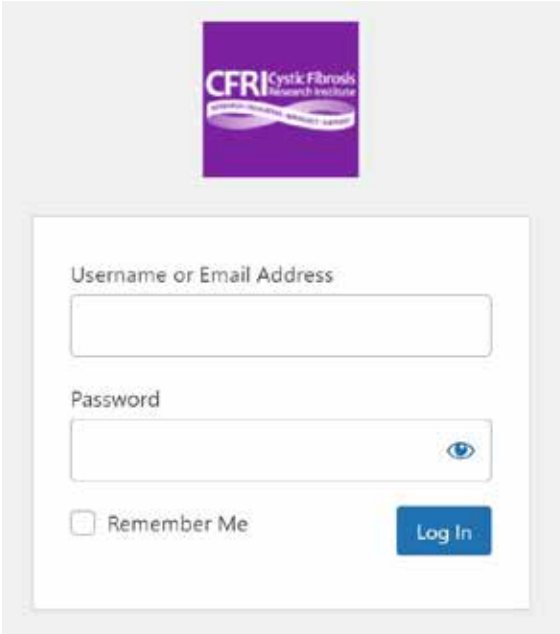
Virtual Attendee Guide

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

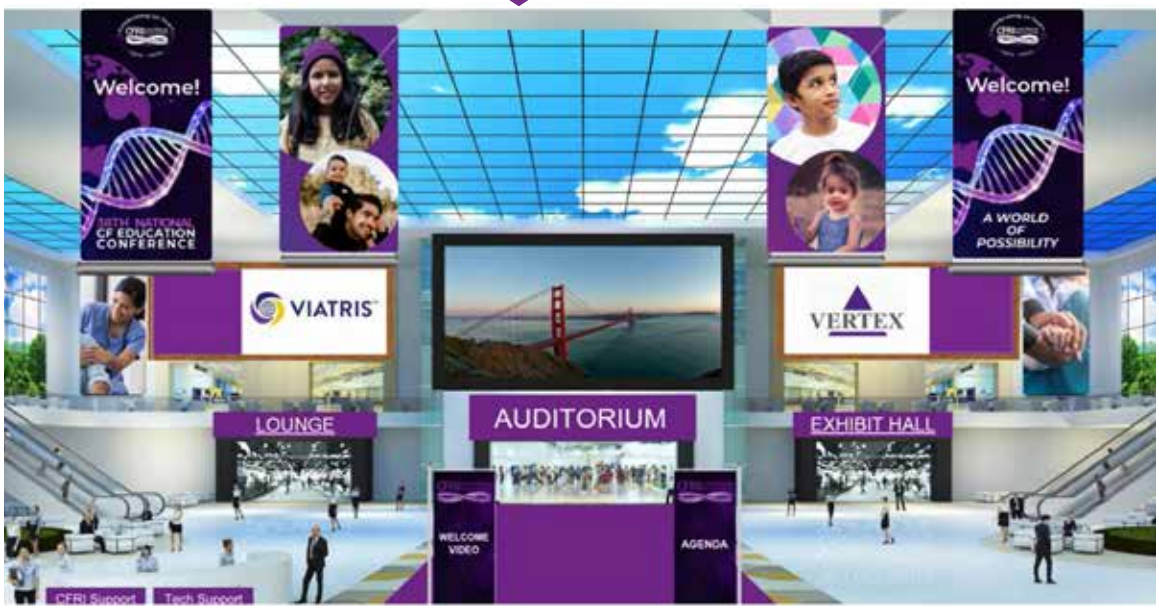
Login Screen

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

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



Main Lobby



In the main lobby, attendees may **use the menu at the top of the screen** to access the Auditorium (watch streamed sessions), Exhibit Hall (visit the virtual booths), and the Lounge (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 38th Annual Education Conference — *A World of Possibility*

Lounge

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

Table Options in the Lounge

The tables in the virtual lounge provide a place to come together and video chat with fellow virtual conference attendees on a shared topic. We offer three tables, each of which can accommodate 8 people at a time. There is no moderator at the table. If no one is there when you drop in, consider returning at a later time.

Optimal table times are:

Friday July 25: 5:30 pm – 7:00 pm Pacific Time (8:30 pm – 10:00 pm Eastern)

Saturday July 26: 7:30 am – 8:45 am Pacific Time (10:30 am – 11:45 am Eastern)
12:35 pm – 1:25 pm Pacific Time (3:35 pm – 4:25 pm Eastern)
4:30 pm – 6:00 pm Pacific Time (7:30 pm – 9:00 pm Eastern)

Table Topics:

Table 1: *Drop In and Say Hello* – No formal topic
Table 2: *I am in the 10% - Unable to use modulators*
Table 3: *My Late CF Diagnosis*

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.

- 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.
- Everyone in attendance is encouraged to wear a mask at indoor events. For those preferring to wear a mask, N95 and surgical masks will be provided.
- Everyone in attendance is required to wear a nametag, which documents that you are registered to attend. If you see someone without a nametag, please let a CFRI staff member know.
- Please refrain from shaking hands to avoid spreading germs.
- If you think you have COVID-19, a cold, virus, or any illness, 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.
- For those with CF, try to maintain the "6-foot" rule to minimize cross-infection risk.
- All participants with CF are required to have completed a sputum culture after June 11, 2025 by a CFF-accredited laboratory and must have a medical release signed by their CF medical team to attend.
- Each person with CF must have their CF physician attest that the person: – has 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.
- 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.
- Do not share cell phones, pens, glasses, plates, or eating utensils.
- Those with CF will be served their meals by hotel personnel or CFRI volunteers and should refrain from touching the serving utensils.
- 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 38th National CF Education Conference

Transformative Sponsor — *Vertex Pharmaceuticals*

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Gold Exhibitor — *Nestlé Health Science*

Bronze Exhibitors — *Digestive Care, Inc.; Clarametyx Biosciences; Walgreens Specialty Pharmacy; 4DMT; Alliance for Patient Access (AfPA)/ Cystic Fibrosis Engagement Network (CFEN); NeilMed; Baxter*

Virtual Exhibitor — *Arcturus Therapeutics*

Organizational Exhibitor — *Emily's Entourage*

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List current as of 07/11/2025. Updates to list available in digital program.

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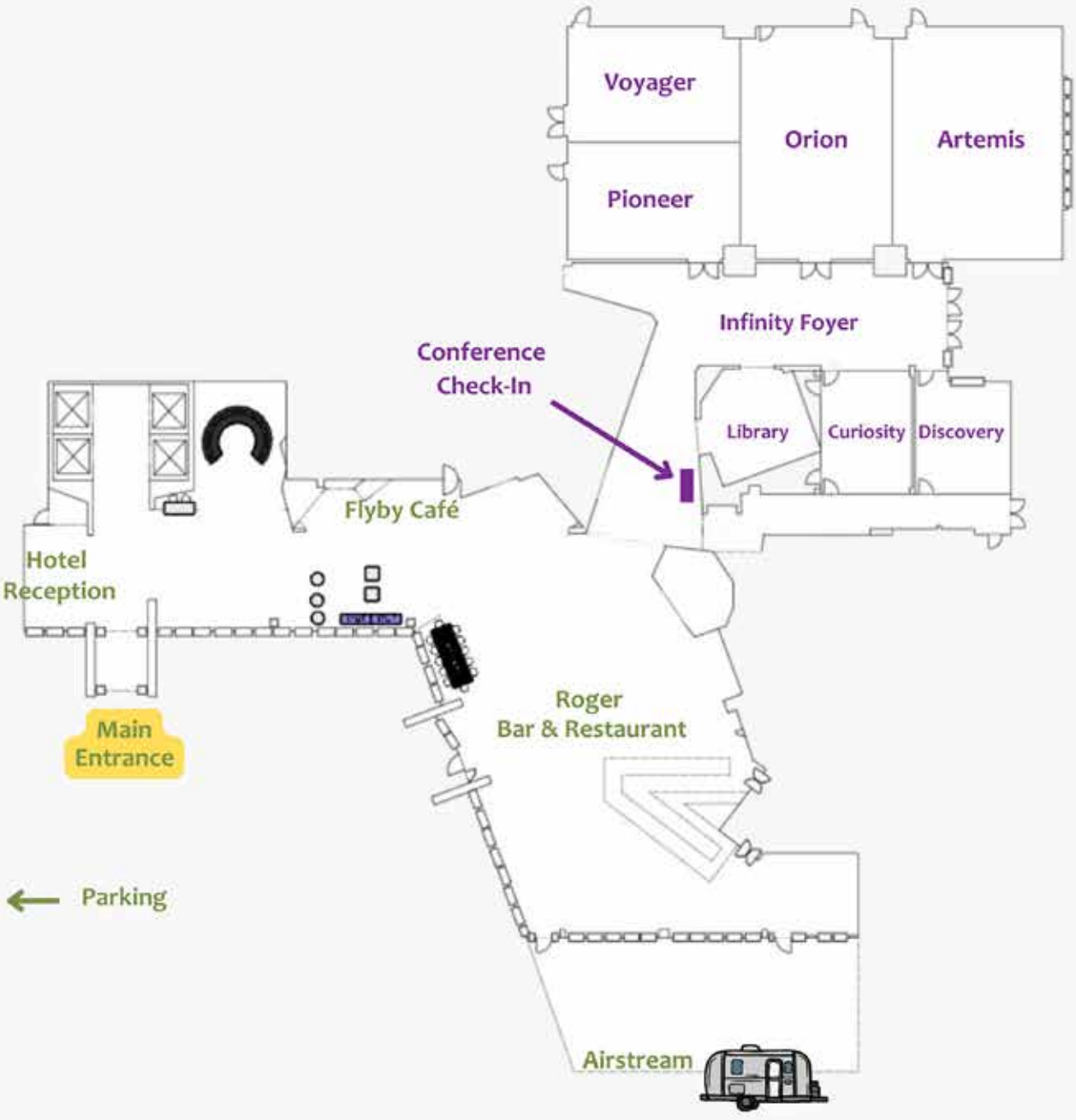

CFRI Board President, Executive Director and Emcees

Bill Hult — CFRI Board President

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

Siri Vaeth, MSW — CFRI Executive Director

Siri Vaeth has been CFRI's executive director since 2018, but her involvement with the organization began soon after her daughter Tess' diagnosis with CF in 1995. As a CFRI volunteer, she raised funds, chaired the Newsletter Committee, and served for 10 years on the Board of Directors. She joined CFRI's staff in late 2013. Siri has a BA in Politics from UC Santa Cruz, and a Master's in Social Welfare from UC Berkeley. She brings many years of nonprofit experience to CFRI, previously serving as executive director of Big Brothers Big Sisters of Santa Cruz County, nonprofit grant writer, United Way campaign associate, and social worker with Migrant Head Start. In addition to serving with several patient advocacy coalitions, Siri is a proud Chair of the American Thoracic Society's Public Advisory Roundtable. Siri's daughter Tess is now 30, and her son Dylan is 26. She lives in Santa Cruz, California.

Devin Wakefield — Saturday Emcee (Research Track)

Devin is a 34-year-old man with cystic fibrosis. He currently is the Research Advisory Committee (RAC) co-chair with two amazing women. Devin also serves on the CF Adult Retreat Committee, where he contributes by finding fascinating speakers from around the globe. He works at Microsoft as an engineer, and lives in Seattle. Devin enjoys going for long hikes in the forest, on the beach, to mountaintops, and around lakes. He does not enjoy coughing up blood, mucus, phlegm, viscous fluids, or petrified mucus plugs.

Rohini McKee — Saturday Emcee (General Session)

Rohini is the mother of two active and fun kids! Ria is 9 and living with cystic fibrosis and Rory is 3 and always making sure his sister is coughing during her treatments and taking her medicine. Rohini and Ria have been involved with CFRI since Ria was 1. On the weekends, you can find Rohini, her husband Richard, and their two kids searching for the best berries at a local farmers market, going on hikes, and watching Bluey.

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

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** denotes CFRI-funded research*

Friday, July 25, 2025

Phages in Progress: Exploring Synergy, Resistance, and Therapeutic Potential*

Friday, July 25, 7:00 pm

Katrine Whiteson, PhD, *University of California Irvine / Irvine, CA*

New strategies to treat antibiotic-resistant infections are urgently needed, especially for people with cystic fibrosis (pwCF) and others with compromised immunity. Phage therapy holds promise as an additional treatment, with recent success stories worldwide, including the use of Pseudomonas phages to treat pwCF. Our team has been collecting phages from Southern California wastewater for nearly a decade and characterizing their genomes, kinetics, and host ranges.

An important limitation of phage therapy is the necessity to match each infection with an effective phage, a process that can be labor-intensive and sometimes unsuccessful. Previously, we described how medium-chain fatty acids (MCFAs) synergize with phages and antibiotics to inhibit *Stenotrophomonas maltophilia*, a common CF pathogen. Here, we expand on this research to include additional key CF pathogens, phages, and antibiotics, aiming to broaden the effectiveness of this combined approach. Notably, combining MCFAs with phages enhances bacterial killing beyond either agent alone, even enabling phage activity against strains previously considered resistant.

We evaluated the inhibitory and synergistic capacity of MCFAs against multiple strains of *S. maltophilia* (18), *Pseudomonas aeruginosa* (14), *Staphylococcus aureus* (18), and *Enterococcus* (91). MCFAs demonstrated broad-spectrum antimicrobial effects across all tested bacteria, showing a concentration dependence correlated with fatty acid chain length. In phage combinations, MCFAs were most effective when some initial phage infectivity was present, but synergy was also observed with previously untreatable strains. Checkerboard assays revealed that MCFAs are synergistic with cell wall-targeting antibiotics (e.g., Polymyxin B), yet less pronounced or even antagonistic with antibiotics that inhibit protein synthesis.

In summary, our findings show that MCFAs substantially amplify the effectiveness of both phage and antibiotic therapies against a spectrum of clinically relevant, antibiotic-resistant pathogens. These results support further development of MCFA-based adjuncts to expand the clinical utility of anti-infective treatments and may expand options for infection treatment in the future.

Dr. Katrine Whiteson is an Associate Professor at the University of California, Irvine, and co-Director of the UCI Microbiome Center, with over 15 years of experience in microbiome research. She earned her BA in Biochemistry from UC Berkeley and her PhD from the University of Chicago. Dr. Whiteson's research focuses on understanding the human microbiome's role in health and disease, particularly in cystic fibrosis (CF). She explores innovative approaches to combatting CF pathogens, including the use of bacteriophages, antibiotics, and small molecule adjuvants. Her lab hunts for phage that can attack CF-pathogens, especially *Stenotrophomonas*, in feces, sewage, sputum and other well-loved samples – the students are brave! Her interdisciplinary work aims to develop tailored therapies for CF patients, with the ultimate goal of improving treatment outcomes and quality of life.

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

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Living Beyond Rare: My Journey with CF, Defying Limits with a Unique Perspective

Friday, July 25, 7:45 pm

Jaelyn Cooper, MHA, *Irving, TX*

This presentation shares the experience of a young woman living with cystic fibrosis – a rare disease made even more isolating by how uncommon it is within her racial community. As an African American patient, Jaelyn Cooper represents a small and often overlooked group within the CF community. Her journey reflects the added challenges of not only managing a complex chronic illness, but also navigating a healthcare system where racial assumptions, gaps in representation, and limited understanding of diversity in CF care continue to exist.

Beyond the medical demands of cystic fibrosis, Jaelyn's story highlights the emotional and social challenges she faced throughout childhood, adolescence, and adulthood - including hospitalizations, disruptions to daily life, and the emotional weight of feeling different and isolated. She also encountered moments where assumptions about her race created added doubt and misunderstanding about her condition, leading to unnecessary challenges in receiving appropriate care.

Despite these obstacles, Jaelyn's journey reflects strength, perseverance, and growth. Through ongoing care, support, and self-advocacy, she continues to overcome challenges while using her experiences to bring attention to the importance of equity, inclusion, and culturally responsive care in the management of cystic fibrosis.

Jaelyn Cooper, MHA, is a proud native of Little Rock, Arkansas. A dedicated advocate and healthcare professional, she was diagnosed with cystic fibrosis (CF) at just 18 months old. Throughout her life, she has navigated the challenges of CF while channeling her experiences into meaningful work within the healthcare field. With a Master of Healthcare Administration and a background in data integrity, policy analysis, and community engagement, Jaelyn is passionate about improving healthcare accessibility and patient outcomes.

In addition to her professional and advocacy work, Jaelyn is deeply committed to fostering connections within the CF community. She understands the importance of representation and shared experiences, using her platform to inspire and uplift others facing similar health challenges. Whether speaking at events, mentoring young individuals with CF, or contributing to discussions on healthcare equity, she strives to be a voice for those who often go unheard.

Beyond her dedication to healthcare and advocacy, Jaelyn enjoys playing instruments, traveling, attending concerts, and roller skating. She finds joy in embracing life's experiences and making the most of every opportunity, demonstrating that living with CF does not limit one's ability to pursue passions and create lasting memories.

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Saturday, July 26, 2025

Addressing Pain in Cystic Fibrosis: Causes, Strategies, and Self-Advocacy

Saturday, July 26, 9:00 am

Nicole Tovar, PT, DPT, *Endurance PT / San Diego, CA*

Pain and musculoskeletal problems are common but under-addressed challenges for people with cystic fibrosis. This presentation highlights key causes of these CF-related issues, including chronic coughing, poor posture, and pelvic floor dysfunction such as urinary incontinence. Physical therapy can offer effective strategies for managing these symptoms through tailored movement, breathing techniques, and pelvic floor rehabilitation.

However, many individuals face barriers to accessing physical therapy, especially when their CF care center does not include a dedicated physical therapist. This session also provides practical tools for self-advocacy, how to request referrals, communicate specific needs, and explore community-based or telehealth PT options.

Nicole Tovar, PT, DPT, has been practicing physical therapy since 2007, after graduating from Columbia University's Doctor of Physical Therapy program. Over the years, she has developed a passion for helping patients regain their mobility and improve their quality of life. From 2012 to 2018, Nicole was a member of the cystic fibrosis and lung transplant team at the University of Southern California, where she worked with patients both during their hospital admissions and in outpatient settings. Her specialized experience in this area allowed her to provide critical care and rehabilitation to individuals navigating complex respiratory challenges. In addition to her clinical expertise, Nicole held a certification in cardiovascular and pulmonary care for 10 years, further enhancing her ability to treat patients with specific needs in these areas. Nicole's commitment to her patients and her extensive clinical experience have made her known for her compassionate care. She currently works in pediatrics in San Diego, California for Specialized Therapy Services.

Elucidating the Ion Transport Functions of CFTR High Expresser Cells (CHEs) and Its Relevance to Intestinal Disease in CF*

Saturday, July 26, 9:00 am

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

Single-cell RNA sequencing (scRNA-seq) studies identified a novel subpopulation of epithelial cells along the rostrocaudal axis of human intestine specifically marked by bestrophin 4 (BEST4) that are enriched for genes regulating pH, GPCR acid-sensing receptors, satiety, cGMP signaling, HCO_3^- secretion, ion transport, neuropeptides, and paracrine hormones. Interestingly, BEST4+ cells in the proximal small intestine express CFTR but have not been linked to the previously described CFTR High Expresser Cell (CHE) subpopulation in rat and human intestine. ScRNA-seq studies in rat jejunum identified CHEs and a gene expression profile consistent with human small intestinal BEST4+ and neuropod cells. Protein immunolocalization confirmed that CHEs express CFTR, BEST4, neuropod proteins, high levels of intracellular uroguanylin (UGN), guanylyl cyclase-C (GC-C), and the proton channel otopetrin 2 (OTOP2), and display long basal processes connecting to neurons. OTOP2, GC-C, and CFTR traffic robustly into the apical domain of CHEs in response to acidic luminal conditions, indicating their roles in luminal pH regulation. Further, rat jejunum organoids expressing CHEs were loaded with the pH sensitive dye pHrodoTM Red AM and examined at varying pH. Maximal fluorescence uptake of the dye was observed in CHEs at pH 5.5 and 6.5 observed in human CF disease. In the ΔF508 cystic fibrosis (CF) rat jejunum, villus and crypt CHEs displayed markedly reduced CFTR mRNA compared to WT, but GUCA2B was

preserved. Apical CFTR protein was lost in ΔF508 CHEs but BEST4 protein was preserved. However, there was an increased abundance of CHE cells in the ΔF508 rat jejunum compared to wild-type animals. Furthermore, ΔF508 rat CHEs expressed higher levels of GC-C at the apical domain compared to wild-type. These data implicate CHEs in intestinal CF disease pathogenesis.

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

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

Getting Older, Growing Bolder: Navigating Aging with CF

Saturday, July 26, 9:55 am

Ahmet Uluer, DO, MPH, *Boston Children's Hospital / Brigham & Women's Hospital / Boston, MA*

The landscape of cystic fibrosis (CF) has been transformed by small molecule CFTR modulator therapies, while those ineligible with rare variants or intolerance to these therapies are also living longer. The growing adult CF population faces a new frontier: aging with a congenital chronic illness marked by persistent inflammation and multisystem involvement. Longevity alone is not the goal; wellness, autonomy, and meaningful quality of life are equally vital. This presentation explores the evolving care needs of adults aging with CF, emphasizing screening, early intervention, maintenance, and individualized approaches that extend beyond CFTR genotype. Age-related comorbidities now affect a significant portion of the CF community, including CF-related diabetes, osteoporosis, gastrointestinal and liver disease, cancer, and cardiovascular disease – many of which overlap with general aging concerns. Additionally, unique challenges such as menopause and hormone replacement for both women and men, hearing loss, vestibular dysfunction, chronic pain, cognitive shifts, and mental health burden must be integrated into long-term care models.

Special attention is given to populations who have undergone lung transplantation and now face complex interactions between immunosuppression, aging, and CF-related complications. The financial, psychological, and existential dimensions of aging with CF – such as navigating careers, insurance, and long-term planning after years of uncertain survival – are also addressed. To meet this moment, we must invest in targeted research, train a new generation of multidisciplinary specialists, and expand programs like the CFRI-sponsored Pat Nash Fellows Program. This presentation calls for a coordinated strategy to ensure that those aging with CF not only live longer but live well.

Dr. Ahmet Uluer is the Director of the Adult Cystic Fibrosis Program and co-Center Director at the combined Boston Children's Hospital and Brigham & Women's Hospital Cystic Fibrosis Center. He is also Director of the Bridges Adult Transition Program at Boston Children's Hospital, providing age-appropriate care and transitional care support to adult survivors of congenital or pediatric acquired

Abstracts & Presenter Profiles

chronic illness. His MPH degree from Harvard TH Chan School of Public Health expanded his interests; he became a member of the CF Foundation (CFF) Global Health Advisory Board and received grant support from the CFF to participate in the International Mentoring Training Initiative (IMTI), specifically to establish CF centers in low-middle income countries. His clinical and research interests are focused on preventing and managing complications of people aging with CF. He is a board member of CFRI and among the founding faculty of the CFRI-sponsored Patrick Nash Fellows Program, mentoring the next generation of providers to care for the aging CF population. He is also the co-host of 'It's a Lung Story' podcast, which explores the realities of aging with CF in the era of modulators and medical advancements.

Optimization of Phage Therapy to Reduce *Pseudomonas*-Induced Inflammation in CF*

Saturday, July 26, 9:55 am

Benjamin Chan, PhD, *Yale University / New Haven, CT*
Kaitlyn Kortright, PhD, *Yale University / New Haven, CT*

People with cystic fibrosis (pwCF) are predisposed to chronic lung infections caused by *Pseudomonas aeruginosa* (PsA), a pathogen increasingly resistant to standard antibiotics. With the growing prevalence of multi-drug resistant (MDR) strains, there is an urgent need for alternative antimicrobial strategies. Bacteriophage (phage) therapy—using viruses that specifically infect and lyse bacteria—offers a promising, targeted approach.

Our work explores how phage therapy not only eliminates bacteria but can also select for bacterial variants that are less virulent or more susceptible to antibiotics. This "evolutionary trade-off" is core to our work and we propose using phages that target key resistance mechanisms or virulence factors in PsA, thereby ensuring that bacterial escape from phage infection results in clinically favorable outcomes.

We present data from a series of assays using bacterial isolates collected from pwCF comparing the effects of sequential phage application versus cocktail-based therapy. Using these isolates, we quantified how each strategy's impact on antimicrobial sensitivity and cross-resistance. Our results suggest that single or sequential phage exposure may drive more predictable and therapeutically useful bacterial changes than cocktails. Furthermore, we demonstrate improved antimicrobial sensitivities in single or sequential phage exposure when compared with cocktails.

Together, these studies demonstrate that phage therapy, when guided by an evolutionary framework, can be both precise and effective. This work lays the foundation for personalized, mechanistically informed phage interventions in CF and other chronic infections, and has direct implications for future clinical trial design.

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

was featured in the Netflix series, "Follow This," as well as in documentaries produced by Vice, Freethink, and BBC One, and has reinvigorated phage therapy in Western medicine.

Dr. Kaitlyn Kortright is a microbiologist specializing in the study of bacterial pathogens and the viruses that infect them, phages. Her work focuses on the interactions between these two microorganisms and the evolutionary consequences of these interactions. With a PhD in Microbiology from Yale University, Dr. Kortright has made significant contributions to the development of phage-based treatments for multi-drug-resistant infections with work bridging microbiology, genomics and therapeutic development.

In addition to her research, Dr. Kortright is committed to mentoring undergraduate and graduate students, supporting students in developing independent research projects and fostering the next generation of scientists. She has published extensively in peer-reviewed journals and presented at international conferences. Dr. Kortright is actively involved in several collaborative research initiatives which aim to understand the basics of phage biology, develop phage as a personalized medicine, and apply phages to address the global crisis of antimicrobial resistance.

Reproductive Health in People with CF

Saturday, July 26, 10:55 am

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

As people with CF are leading longer and healthier lives, sexual and reproductive health has become an increasingly important topic with a number of unique questions. It is critical that we provide high quality evidenced-based data to men and women with CF on topics including contraception, fertility, pregnancy and lactation, and assisted reproductive technology options. More females are experiencing pregnancy and more males are seeking assisted reproductive technology options with a large population of people with CF wanting to become parents overall. This session will highlight what is known and unknown about male and female infertility in CF, contraception and its impact on health in CF, pregnancy, and lactation in the era of widespread use of CFTR modulators.

Dr. Raksha Jain is a Professor of Pulmonary and Critical Care Medicine at the University of Texas Southwestern Medical Center. She completed her Master's of Science in Clinical Investigation at Washington University and joined the faculty at UT Southwestern in 2010, where she serves as Medical Director of the CF and Bronchiectasis Program.

Dr. Jain focuses much of her research on women's health. She has been working to understand the etiologies behind the sex disparity observed in CF and other airway diseases using a translational approach with a focus on the role of estrogen. Her interest in estrogen led to studies of pregnancy, where she is co-leading a large investigator initiated multicenter prospective study of pregnancy in females with CF (MAYFLOWERS). She has been awarded multiple CF Foundation and NIH grants and is fostering studies of women's health in the larger community by serving as Chair of the CFF TDN Sexual health, reproduction and gender research (SHARING) working group

Dr. Jain has also made significant contributions to therapeutic development for people with CF. She has led multiple local and investigator-initiated and industry clinical trials in CF and is serving on the CFF clinical research executive committee.

Abstracts & Presenter Profiles

Designing a CF Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cell-Derived Ionocytes*

Saturday, July 26, 10:55 am

Steven Jonas, MD, PhD, *University of California Los Angeles / Los Angeles, CA*
Ruby Sims, PhD, *University of California Los Angeles / Los Angeles, CA*

Gene therapies that restore CFTR function would offer life changing cures for CF patients. A key challenge to realizing these therapies is the limited ability to identify and then target specific stem cells responsible for CFTR protein expression in the human airway. One such stem cell population, ionocytes has been identified as an opportune target for engineering CFTR expression due to their apical location and normally high level of CFTR expression. Here we present the development of broadly applicable nanoscale tools required to restore function of CFTR protein production towards a clinically translatable mRNA therapy. Lipid nanoparticles (LNP) were designed as delivery vehicles for these genetic engineering reagents and initially utilized for the delivery of codon-optimized gene editing tools to enable site specific integration of a CFTR donor template, demonstrating complete restoration of CFTR protein production and function in 16HBEgeG542x cells with just 3.5% CFTR integration. These technologies were then applied to encapsulate and deliver mRNA constructs encoding for CFTR towards inducing transient expression in shorter-lived ionocytes. Through further optimization of ionizable, sterol and pegylated lipids this LNP platform was successfully translated to Airway Basal Stem Cells (ABSCs), transfecting up to 60% of treated cells with mRNA encoding for an engineered green fluorescent protein reporter, and facilitating the transition to differentiated ABSC ALI. To address the non-specific targeting of ionocyte stem cell populations, single cell RNA sequencing data was mined to identify ionocyte surface proteins/receptors targetable by monoclonal antibodies, and protocols validated to enable their click chemistry conjugation to the surface of LNPs (Ab-LNPs). While the transfection of differentiated ABSCs remains challenging, we have developed in vitro models that enable rapid screening Ab-LNP formulations, utilizing submucosal gland Matrigel organoid models containing ionocyte-directed cell types during differentiation to replicate structure and function of the airway. It is through the development of these nanotechnologies that we provide the nucleic acid constructs, delivery vehicle, and in vitro models required for validation, setting the stage for developing clinically translatable mRNA therapies that offer a definitive cure for CF patients.

Steven J. Jonas, MD, PhD, is an Assistant Professor of Pediatrics at the University of California, Los Angeles (UCLA) David Geffen School of Medicine, and an investigator at UCLA's California NanoSystems Institute and Broad Stem Cell Research Center. His laboratory explores new ways to probe and engineer cells that leverage advances in microfluidics, nanofabrication, and gene editing approaches. In his clinical practice, he serves as an attending physician at UCLA Mattel Children's Hospital where he directs the care of patients with a variety of hematologic and oncologic conditions as well as those undergoing hematopoietic stem cell transplantation and/or gene therapy. He also serves as the Associate Program Director of the UCLA Pediatric Hematology/Oncology Fellowship Program where he strives to foster an inclusive scientific training environment that guides the next generation of pediatric hematology/oncology physicians as they embark on their research journeys.

Dr. Jonas' research explores the intersection of pediatrics, hematology/oncology, stem cell biology, bioengineering, and nanoscience and nanotechnology. His laboratory harnesses advances in these areas to develop and apply new, broadly applicable and accessible technologies and methodologies that support the children's health and regenerative medicine research communities along two themes: i) developing efficient, high throughput, and economical gene and cell therapy manufacturing platforms; and ii) fueling precision pediatric oncology via the translation of liquid biopsy methods that can help amass the data required for longitudinal and real time molecular profiling of pediatric malignancies and

monitoring their response to treatment dynamically. These capabilities advance the laboratory's efforts to create tools that enable stem cell biologists to probe and interact with stem cells more precisely and empower clinician scientists to apply this knowledge to design and implement new treatments more quickly. By engaging in these efforts, his team is helping to accelerate the discovery and democratize the implementation of emerging gene and cellular therapeutic strategies and precision medicine-focused diagnostic approaches that will unlock new solutions for impacting the care of children with complex healthcare needs. Dr. Jonas has received several awards for his research, including the 2024 Society of Pediatric Research Young Investigator Award, a Director's Early Independence Award from the National Institutes of Health and has been the recipient of Young Investigator and Scholar awards from the Alex's Lemonade Stand Foundation for Childhood Cancer Research, the Hyundai Hope on Wheels Foundation, and the Tower Cancer Research Foundation.

Dr. Ruby Sims is a postdoctoral scholar in the Department of Pediatrics at UCLA's David Geffen School of Medicine. Under the guidance of her mentor Dr. Steven J. Jonas, Dr. Sims leads research projects at the interface between engineering, materials science and cell biology to develop nanoscale tools for next-generation, precision medicine gene and cellular based therapies. As an Elizabeth Nash Memorial Fellowship awardee, her work with the CFRI is focused on advancing lipid nanoparticle-based solutions for the safe, effective, targeted delivery of gene editing machinery towards accessible, one-time curative treatments for monogenic disorders. In collaboration with her colleagues, these nanotechnology tools have elucidated a deeper understanding of stem cell gene editing mechanisms and provide opportunities to improve the development, and implementation of cell and gene therapies.

Dr. Sims' research is supported by the CRFI's Elizabeth Nash Memorial Fellowship and the Alex's Lemonade Stand Foundation for Childhood Cancer Research's Young Investigator Award.

Increased Risk of GI and Other Cancers in People with CF

Saturday, July 26, 11:50 am

Steven Freedman, MD, PhD
Beth Israel Deaconess Medical Center / Harvard Medical School / Cambridge, MA

With advances in the care of patients with CF, have come new challenges. As people with CF age, we are seeing the emergence of an increase in cancers. These are concentrated in the GI tract, but other cancers are seen including breast, lung, and cervical cancer. The reason for the increased cancer risk in CF is not known but a number of factors predispose. First, CFTR itself is an anti-oncogene (tumor suppressor). Published data shows that CFTR interacts with the tumor suppressor PTEN to activate PI3 kinase, regulate the inflammatory response, and activate host defenses against *Pseudomonas*. With loss of CFTR function, loss of PTEN leads to a pro-oncogenic and hyperinflammatory state along with impaired *Pseudomonas* clearance.

A number of other predisposing factors for GI tract cancers include the high saturated fat diets, the gut dysbiosis, altered GI transit times, and chronic GI inflammatory state that are all linked to an increased risk of colorectal cancer. For pancreatic cancer, diabetes is a risk factor and thus taken together along with the loss of CFTR tumor suppressor function, we have a perfect storm for the development of malignancy. A number of questions remain: Will CFTR modulators have a protective effect on malignancy? Can we attenuate risk with a 'healthier' diet? Is the biologic behavior of tumors in CF more aggressive? How do we screen for malignancies with the least invasive procedures? Should we treat cancers in people with CF similar to non-CF patients?

Abstracts & Presenter Profiles

Steven Freedman, MD, PhD, is Director of the Pancreas Center at Beth Israel Deaconess Medical Center, Chief of the Division of Translational Research, and Professor of Medicine at Harvard Medical School. He has played a leadership role in clinical/translational research across Harvard through his prior role as the Associate Dean for Clinical and Translational Research and Co-Director of the Harvard CTSA (Harvard Catalyst). He is Director of the Grant Review and Support Program, a unique longitudinal program that provides project management support and grant writing tools to enhance the transition from an NIH K to R01 grant for junior faculty across Harvard.

Dr. Freedman's expertise is in exocrine pancreatic disease with a focus on pancreatitis, pancreatic cancer, pancreatic enzyme development and cystic fibrosis as well as diseases of premature infants with a translational research focus on fatty acid metabolism.

He helped establish the CF Foundation funded DIGEST program to train pediatric and adult gastroenterologists in the GI aspects of CF and plays a leadership role for the CF Foundation to design, develop and carry out GI-related CF research.

Characterization of Ionocyte Subtypes in Cystic Fibrosis Ferrets*

Saturday, July 26, 11:50 am

Feng Yuan, PhD, *University of Alabama / Birmingham, AL*

Background:
Pulmonary ionocytes express high levels of CFTR mRNA and protein within an apical cap in the airway epithelium. A better understanding of pulmonary ionocyte-specific CFTR-mediated Cl- secretion and absorption will provide the foundation for enhancing the effectiveness of therapeutic approaches. Furthermore, relatively little is known about the phenotypic changes occurring in pulmonary ionocyte subtypes in CF.

Methods:
FOXI1-CreERT2::CFTRL/L (CFTR-cKO) ferrets were used for ionocyte-specific CFTR deletion studies. CFTRG551D/G551D ferrets were used to model the CF phenotype following the removal of the CFTR potentiators (VX-770) from their rearing protocol.

Results:
To study absorptive Cl- currents (i.e., the movement of chloride from the air-liquid interface culture apical side to the basolateral side), we applied asymmetrical high-apical/low-basolateral Cl- gradient conditions. Both CFTR-cKO and CF epithelium unequivocally lacked absorptive CFTR-dependent Cl– currents, which were induced by forskolin/IBMX and inhibited by GlyH101 under high apical and low basolateral Cl– conditions. To determine if ionocyte-mediated Cl– secretion relies on CFTR, we evaluated ionocyte CFTR-cKO and CF epithelium under low-apical/high-basolateral Cl– gradient conditions. The short circuit current change in response to forskolin/IBMX and GlyH101 treatments demonstrated a similar magnitude of alteration in CF (absent) and CFTR-cKO (~50-70% reduced) cultures compared to controls. FOXI1-lineage tracing and ionocyte localization in wild-type (WT) ferrets revealed a gradient of ionocyte abundance from proximal to distal regions of the large conducting airways (nasal mucosa > trachea > intrapulmonary bronchi). The CF disease state induced substantial expansion of ATP6V1G3+ and BSND+ ionocytes within the intrapulmonary airway SMG ducts. The primary driving force for ion secretion in fish ionocytes is the sodium-potassium chloride co-transporter (NKCC1). Our in vivo ionocyte lineage tracing demonstrated that NKCC1 was localized to FOXI1-lineage ionocyte basolateral and tubular network membranes. The CF proximal trachea showed two types of ionocytes: NKCC1+ATP6V1G3+ and NKCC1+ATP6V1G3-.

Conclusion:
Pulmonary ionocytes mediate CFTR-dependent chloride absorption and secretion. Ionocyte ion channel expression or subtypes are highly sensitive to CF, indicating that these specialized cells and rare cell progenitors can adapt to the altered CF environment to maintain proper physiological function.

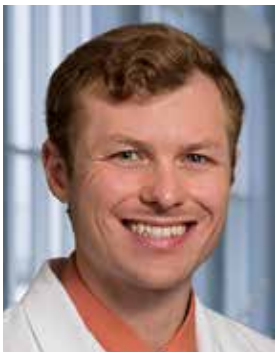
Dr. Feng Yuan earned his PhD from Zhongshan School of Medicine at Sun Yat-sen University. During his doctoral training, he focused on ion channel properties and their roles in biological and diseased processes. In 2017, he began his postdoctoral training in cystic fibrosis lung disease with Dr. John Engelhardt at the University of Iowa. His work demonstrated that pulmonary ionocytes mediate air surface liquid absorption and secretion via CFTR-dependent chloride and bicarbonate transport. In 2024, Dr. Yuan received the Cystic Fibrosis Research Institute's New Horizons Award and the Cystic Fibrosis Foundation Spring Research Grant. Starting in December 2024, Dr. Yuan joined the Department of Medicine, Pulmonary Division at The University of Alabama at Birmingham as an assistant professor on the tenure track.

Emerging Cardiovascular and Metabolic Risk Factors in the Era of Highly Effective CFTR Modulators

Saturday, July 26, 1:45 pm

Gregory Ratti, MD, *University of Texas Southwestern / Dallas, TX*

With the advent of highly effective CFTR modulators, people with cystic fibrosis have experienced remarkable improvements in their health in the form of improved pulmonary function, reduced exacerbations, and weight gain. This weight gain has resulted in a dramatic increase in the number of people meeting BMI criteria for overweight and obesity. As of the 2023 CF Foundation Patient Registry Report, 28% of people were classified as overweight and 13% as obese, compared to 17% meeting these combined criteria in 2003. Recognition of this problem has resulted in a change in the recommendations for dietary counseling for individuals with CF to a more individually-tailored approach based on the person's BMI. In addition, modulators have been associated with modest increases in blood pressure and changes in lipid profiles. The emergence of obesity, dyslipidemia and hypertension may contribute to the development of metabolic syndrome which is associated with increased cardiovascular risks in the general population. As people with CF live longer and healthier lives, it will be important to recognize these emerging cardiovascular and metabolic risk factors and address them early to prevent future complications. In this presentation we will review available literature examining the metabolic and cardiovascular changes that occur in individuals exposed to CFTR modulators and discuss interventions to improve the overall health of people with CF.



Dr. Gregory Ratti started his medical training at the University of Cincinnati where he first gained interest in caring for people with cystic fibrosis. He then went on to complete internal medicine training at Barnes Jewish Hospital/ Washington University in St. Louis before completing his Pulmonary and Critical Care Fellowship at the University of Texas Southwestern in Dallas, TX. Here he was able to continue to follow his interests when he joined the Adult CF clinic at UTSW where he cares for people in both the inpatient and outpatient settings. In addition to routine care for these patients, he has a particular interest in the metabolic effects of highly effective modulators on people with CF. In addition to caring for people with CF, he serves as the medical director of the MICU at Clements University Hospital. Outside of medicine Dr. Ratti enjoys spending time cycling, fly fishing, and spending time with his two young children.

Abstracts & Presenter Profiles

A Hybrid Gene Correction Strategy for Cystic Fibrosis*

Saturday, July 26, 1:45 pm

Anais K. Amaya, PhD, *Stanford University / Palo Alto, CA*
Matthew Porteus, MD, PhD, *Stanford University / Palo Alto, CA*

Highly effective modulator therapy (HEMT) has dramatically improved the quality of life of people living with CF (pwCF). However, HEMT is not available to all pwCF, particularly those with mutations that result in missing or incomplete protein. Moreover, pwCF eligible for HEMT do not always respond to treatment or have access to it. Improvements in life expectancy are not reflected in global statistics because pwCF born in low and middle-income countries have virtually no access to these transformative medications.

Knowing what the genetic defect is gives us the ability to repair it at the DNA level. Therefore, we aim to develop a durable curative therapy that corrects the genetic defect directly at the affected locus and maintains endogenous regulation. Our group previously developed CRISPR/Cas9 ribonucleoprotein (RNP) and AAV reagents for efficient ex vivo editing of airway basal cells. However, these methods require transplantation of edited cells into the airways, which remains challenging despite proof-of-concept work in the trachea. Therefore, our goal is to create an approach that could be used for editing directly in the lung, either ex vivo or in vivo. Editing directly in the lung adds complexity as the right cell type, basal cells (the stem cells of the lung), must be corrected to achieve durable effects.

In this study, we describe our efforts to develop viral and non-viral editing reagents to correct the most common CF-causing mutation in human bronchial epithelial cells and precision-cut human lung slices. There are two components of the correction system that we need to deliver: the CRISPR/Cas9 nuclease with the sgRNA and the donor template to be used by the cell to correct the genetic defect. We evaluated various delivery strategies including lipid nanoparticles (LNPs), virus-like particles, lentiviral vectors, adeno-associated viral vectors (AAVs), polymeric nanoparticles (NPs), and cell-penetrating peptides (CPPs) for delivery to lung slices. We have identified viral and non-viral vectors, including LNPs, NPs, and AAVs, that efficiently deliver nucleic acids to lung cells in culture and basal cells within lung slices. These reagents could later be used towards ex vivo or in vivo editing and enable clinical translation.

Dr. Anais Amaya is a Postdoctoral Research Fellow at Stanford's Department of Pediatrics, specializing in gene therapy for inherited diseases like cystic fibrosis. She earned her PhD from the University of Sydney, developing CRISPR/Cas9 therapies for pediatric genetic liver diseases.

Venezuelan-born and educated at Universidad Simón Bolívar and Lund University, Dr. Amaya has received numerous awards including the Swedish Institute Scholarship, the Panos Ioannou Young Investigator Award from the Australasian Gene and Cell Therapy Society and Stanford Maternal & Child Health Research Institute Postdoctoral Award. Her research focuses on developing safer delivery vectors for gene therapy to create curative treatments.

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

Dr. Porteus received his undergraduate degree at Harvard in History and Science where his honors thesis studied the recombinant DNA controversy of the 1970s. He then completed his MD and PhD training at Stanford, clinical

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

Living to Dream or Fighting to Breathe? Why Cystic Fibrosis Isn't Equal for All

Saturday, July 26, 2:40 pm

Meghan McGarry, MD, MAS, *University of Washington / Seattle, WA*

Treatments for cystic fibrosis have transformed the disease, leading to significantly improved outcomes for people with CF. However, not all groups of people with CF have benefited equally from these advances in CF care and treatments. Certain groups of people with CF face worse outcomes, including severe lung disease, increased risk of pulmonary infections, increased risk of CF-related diabetes, and higher mortality. These health disparities are not explained by CFTR genetic severity, diagnosis age, or socioeconomic status. There remains a lot to learn about what is contributing to these disparities. Most CF clinical trials included very few people who were of a race or ethnicity other than non-Hispanic white. Genetic testing and newborn screening often did not screen for CFTR variants that occur in all groups, leading to some groups having delayed diagnoses. Unequal access to novel therapeutics such as CFTR modulators has widened existing health disparities. Even lung transplantation is not equally available to all people with CF. The drivers of health disparities in CF are likely multifactorial and involve biases in care, the environment a person lives in, community differences, education, language barriers, and economic stability. We will discuss what further research and actions are needed to advance care and improve outcomes for all people with CF.

Dr. Meghan McGarry is a Pediatric Pulmonologist and Associate Professor of Pediatrics at the University of Washington and Seattle Children's Hospital. She has formal training in clinical pharmacology, epidemiology, and clinical research. One area of Dr. McGarry's research is investigating health disparities in pulmonary disease severity in the Latino cystic fibrosis (CF) community. She has found that Latino children with CF have lower lung function and are at increased risk for pulmonary infections early in childhood. She is focused on determining what contributes to lung disease severity and why some children become sicker than others. Also, Dr. McGarry works to improve newborn screening for cystic fibrosis and diagnosis for all people with CF. She is the lead author of the new Cystic Fibrosis Foundation guideline for CF newborn screening. Dr. McGarry advocates for access to affordable CF therapies for all people with CF across the world. Dr. McGarry's mission is to improve the health and quality of life for all those with cystic fibrosis through research, education, advocacy, and community engagement.

Abstracts & Presenter Profiles

Corrected CFTR-F508del is Targeted for Endolysosomal Degradation*

Saturday, July 26, 2:40 pm

Ron Kopito, PhD, *Stanford University / Palo Alto, CA*
Celeste Riepe, PhD, *Stanford University / Palo Alto, CA*

Over 80% of people with cystic fibrosis (CF) carry the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR), a chloride ion channel at the apical plasma membrane (PM) of epithelial cells. F508del impairs CFTR folding, causing it to be destroyed by endoplasmic reticulum associated degradation (ERAD). The principle therapeutic strategy for cystic fibrosis is oral administration of small-molecule correctors that act as pharmacological chaperones to help CFTR-F508del fold at the ER and traffic to the PM. However, corrected CFTR-F508del is degraded more rapidly in post-ER compartments than wildtype CFTR, reducing the efficacy of the drugs. We performed a CRISPR/Cas9 knockout screen to identify protein quality control (PQC) machinery that targets CFTR-F508del in the presence of two correctors, elexacaftor and tezacaftor, that are principal components of the CF drug therapy, TRIKAFTA®. Corrected CFTR-F508del was fully stabilized by treatment with TAK-243, a potent and selective inhibitor of ubiquitin activation, indicating that its degradation is mediated exclusively by ubiquitin-dependent pathways operating in both ER and post-ER compartments. Our screens with correctors uncovered components of the lysosomal V-ATPase (ATP6V0C, ATP6V1E1, and ATP6V1F), late endocytic machinery (VSP11 and RAB7A), machinery important for trafficking of lysosomal enzymes (GNPTAB and IGF2R), and RFFL, an E3 ligase previously implicated in corrected CFTR-F508del degradation at the PM. Unexpectedly, we observed that knocking out the gene encoding MYLIP/IDOL, an E3 ligase previously implicated the endolysosomal degradation of the low-density lipoprotein receptor (LDLR), stabilized corrected CFTR-F508del, revealing an previously undescribed role of the liver X receptor cholesterol efflux pathway in modulating corrected CFTR-F508del stability. Our data suggest that development of pharmacological inhibitors against the endolysosomal system can enhance the efficacy of current CF therapeutics.

Dr. Ron Kopito is an American cell biologist renowned for his pioneering work on protein degradation and its implications for human disease, particularly cystic fibrosis (CF). A professor at Stanford University, Dr. Kopito's research has been instrumental in uncovering how misfolded proteins are recognized and degraded within cells.

Dr. Kopito's long association with CF began in the early 1970s when Dr. Harry Shwachman, offered him a summer job as a high school student doing sweat tests in his laboratory at Boston's Children's Hospital. In the early 1990s, Dr. Kopito's lab provided crucial insights into the fate of ΔF508-CFTR, establishing that misfolded CFTR is recognized by the cellular quality control machinery in the endoplasmic reticulum and targeted for degradation via the ubiquitin-proteasome system, rather than reaching the cell membrane where it is needed for ion transport. This discovery helped shift the focus of CF research toward understanding and manipulating protein folding, stability, and trafficking, paving the way for the development of small-molecule modulators, a strategy that underlies modern CF treatments, like lumacaftor and elexacaftor. Dr. Kopito's broader work on protein homeostasis has also influenced research into neurodegenerative diseases and other protein misfolding disorders.

Celeste Riepe, PhD, is a senior postdoctoral fellow in the Kopito laboratory in the Department of Biology at Stanford University. She specializes in using state-of-the-art genetics and genome editing methods to better understand why certain people with cystic fibrosis fail to respond to modulator therapies like TRIKAFTA. Specifically, Dr.

Riepe works to conduct genome-wide CRISPR/Cas9 screens to discover drug targets that will improve the trafficking and function of corrector-resistant cystic fibrosis transmembrane conductance regulator (CFTR) variants. A native of San Marcos, TX, Dr. Riepe received a Bachelor of Arts in Biochemistry and Cell Biology from Rice University, and a PhD in Molecular and Cell Biology from the University of California, Berkeley, where she worked in the laboratories of Nicholas Ingolia and Jacob Corn to characterize changes in protein synthesis and ribosome composition after Cas9-mediated genome editing. As a postdoctoral researcher, Dr. Riepe has been awarded the CFRI Elizabeth Nash Memorial Fellowship and the CFF Path-to-a-Cure Postdoctoral Fellowship for her work on CF.

Nucleic Acid-Based Therapies for Cystic Fibrosis: Progress and Challenges

Saturday, July 26, 3:40 pm

Joseph Pilewski, MD, *University of Pittsburgh / Pittsburgh, PA*

Effective CFTR modulators have had a dramatic impact on lung disease in CF. However, not all people with CF are eligible, some are intolerant, and some have less benefit than others. The need for new therapies and ultimately a cure for CF in the lung and other organs remains significant and is a high priority for the CF community. Nucleic acid-based therapies (NABTs) are designed to restore or correct the CF gene defect and are a form of gene therapy. Current approaches include recombinant viruses (AAV, HSV, lentivirus) to deliver a normal copy of the CF gene to airway cells, lipid nanoparticles (like the those used for COVID-19 vaccines) to deliver a normal CFTR messenger RNA, and anti-sense oligonucleotides to overcome defects in specific CF genes. Research with these approaches has progressed from laboratory studies to early phase clinical trials focused on specific populations to assess safety and efficacy. This presentation will review the basic biology and approaches to NABTs for CF, discuss the challenges with designing and executing clinical studies of NABTs, and consider the future of these therapies and gene editing approaches that are under development.

Dr. Joseph Pilewski is a Professor of Medicine at the University of Pittsburgh and the Associate Chief for Clinical Affairs, Pulmonary, Allergy, Critical Care and Sleep Medicine at the University of Pittsburgh Medical Center. He specializes in cystic fibrosis and lung transplantation. He completed medical school at the University of Rochester and residency and fellowship at the Hospital of the University of Pennsylvania. Dr. Pilewski's research interests mirror his clinical interest and expertise. He previously directed a research program in epithelial cell biology focused on ion transport and gene transfer and has contributed to translational research projects and clinical trials focused on development of new therapies for CF and lung transplant. He is a co-investigator on NIH and CF Foundation sponsored grants, and has served in leadership roles for the Cystic Fibrosis Foundation Therapeutics Development Network.

Optimization of Activity and Improved Delivery of Bacteriophages Targeting *Burkholderia spp**

Saturday, July 26, 3:40 pm

Daria Van Tyne, PhD, *University of Pittsburgh / Pittsburgh, PA*

Antibiotic resistance presents a deadly problem for people with cystic fibrosis (pwCF), who are frequently treated with antibiotics throughout their lives. One particularly problematic pathogen in pwCF is *Burkholderia cepacia complex* (Bcc) bacteria. BCC are often intrinsically antibiotic-resistant and can cause chronic infections in pwCF. High mortality and a lack of effective antibiotics for pwCF with Bcc infections highlight the urgent need for new treatment options. Bacteriophage (phage) therapy, or the use of viruses that target bacteria to treat infection, is a promising alternative therapeutic approach to treat antibiotic-resistant infections, including in pwCF. The Van Tyne lab has identified and characterized several Bcc-targeting phages, including both lytic phages and prophages isolated from the genomes of

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of clinical Bcc isolates. We received requests for phage therapy evaluation from nearly 40 patients (nearly all pwCF) and evaluated the susceptibility of over 100 clinical Bcc isolates against a panel of six Bcc-targeting phages. We observed that over two-thirds of all isolates tested were susceptible to at least one phage in our collection, but there were differences in phage susceptibility between different Bcc species. One prophage-derived phage called BCC02 was very broadly active, being able to plaque on over half of all isolates. While this prophage-derived phage is not immediately useful clinically, it could be converted to a lytic phage through experimental evolution, chemical mutagenesis, or genetic manipulation. Finally, because phage therapy in pwCF is often delivered via nebulization, we tested the viability of a lytic phage called Bch7 that was previously used in phage therapy to treat a lung transplant patient with CF. We quantified the viability of Bch7 after undergoing aerosolization in jet and vibrating mesh nebulizers, and also assessed the distribution of phage-containing aerosols using a next-generation impactor. In line with prior studies, we observed an 80-95% loss of phage viability after nebulization, though a vibrating mesh nebulizer resulted in the least amount of loss. Taken together, this work increases our understanding of Bcc-targeting phages and can inform the development of phage therapy for the treatment of Bcc infections in pwCF.

Daria Van Tyne, PhD, is an Associate Professor of Medicine in the Division of Infectious Diseases at the University of Pittsburgh School of Medicine. Dr. Van Tyne completed her undergraduate work at Vassar College, followed by PhD studies at the Harvard T.H. Chan School of Public Health. She then pursued post-doctoral training at Harvard Medical School and started her research lab at the University of Pittsburgh in 2018. The Van Tyne lab studies how bacteria evolve during human infection using functional genomics approaches. The lab is also developing bacteriophage therapy to treat antibiotic-resistant infections, with a primary focus on bacterial pathogens that are problematic for people with CF. Dr. Van Tyne is a member of the University of Pittsburgh Center for Innovative Antimicrobial Therapy, an organizing member of the Center for Evolutionary Biology and Medicine, and she is the Co-Chair of the Pittsburgh Phage Program.

Sunday, July 27, 2025

Panel: Mental Health Impacts of CF Upon the Family

Sunday, July 27, 9:30 am

Deborah Menet, LCSW – Moderator, *Stanford CF Center / Palo Alto, CA*
Jacob Fraker, MSW, *Sacramento, CA*
Sara Modlin-Tucker, DO, *Novato, CA*
Hema Patel, *Mission Viejo, CA*
Gail Wright, RN, *Pleasant Hill, CA*

The emotional challenges of cystic fibrosis extend beyond the person diagnosed with the disease, and extend to the entire family system, including parents, siblings, partners, spouses, and children. The TIDES study (Quittner) documented the high rates of depression and anxiety experienced by those with CF and their caregivers. Notably, mothers had higher rates of anxiety that their children with CF. A diagnosis of cystic fibrosis places emotional stress upon the family system, altering communication between partners/spouses, and often leading to very different parenting practices between the child with CF and their "well" sibling. The daily health regimen and hospitalizations can disrupt routines and exacerbate stress for all members of the family. Couples may have differing opinions related to their children's CF care, including infection control and adherence to the daily medical regimen. Siblings of those with CF may hide their own issues, as they internalize that they should not draw parents' attention

away from their sibling. The challenges of CF may also lead to resilience among family members, strengthening bonds and shifting priorities. In this panel discussion, facilitated by a CF social worker, we will hear about the experiences of varied members of the family system, including an adult with CF, a sibling, a mother, and a spouse. Each will share the diverse impact that CF has had upon the family system and upon their own lives.

Moderator and Panelists

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

Jacob Fraker, MSW, was born and raised in the small rural town of Sonora, CA. Jacob was diagnosed in utero with cystic fibrosis after his older brother was diagnosed at age 3 due to salty sweat. Jacob used to travel 3 hours from Sonora to San Francisco to get care until moving to San Jose for his Undergrad. Jacob received his Bachelor's in Social Work at San Jose State University. During his time there, he served as the President of the Undergraduate Social Work Association. In 2017, following a legislative fellowship, Jacob began working CFRI on rare disease policy issues. Jacob then went on to UC Berkeley where he got his Master's in Social Welfare in their Strengthening Organizations and Communities track. During his time at UC Berkeley, Jacob interned with CFRI, helping establish a rare disease presence in the State Capitol, and went on to intern in the office of the then Assemblymember Susan Eggman. Jacob carried bills, staffed the member in policy committee, and advised on Health policy. Following his graduation, Jacob went to work at the Governor's Office of Emergency Services as a Health and Housing Recovery Specialist before being asked back to the Capitol to join then Senator Eggman's team as the Senate Consultant to the LGBTQ Caucus. Currently, Jacob works with the new Vice-Chair of the LGBTQ Caucus, Senator Caroline Menjivar. In this role, Jacob advises 14 state legislators on broad issues relating to the LGBTQ+ Community. He manages their legislative and budget portfolio, implements the Caucus departmental oversight, and supports the Caucus on its efforts to build the bench for the future LGBTQ leaders in California.

Additionally, Jacob currently serves as the executive director of the California LGBTQ Foundation, a nonprofit dedicated to uplifting and supporting the LGBTQ+ community through scholarship, research, and community building. He is also a current board member of the Sacramento LGBT Center.

Sara Modlin-Tucker, DO, is a dual board-certified physician in Family Medicine and Neuromusculoskeletal Medicine. This career trajectory started from her life experience growing up with a sister with cystic fibrosis. She has been immersed in the CFRI community her whole life through the roles of her parents and sister's involvement with CFRI. As well as her own professional career, she has been involved in cystic fibrosis research. She has attended numerous CFRI conferences and retreats and has been an advocate for the CF community.

Abstracts & Presenter Profiles

She currently serves as Vice Chair of the Osteopathic Manipulative Medicine Department and Fellowship Director at Touro University California, where she is passionate about teaching the next generation of osteopathic physicians. She earned her Doctor of Osteopathic Medicine from Touro University California in 2017 and completed both her Family Medicine and Osteopathic Neuromusculoskeletal Medicine (ONMM) residencies at Maine-Dartmouth Family Medicine Residency in Augusta, Maine. She holds a bachelor's degree from the University of California, Santa Cruz.

Her sister's life continues to be a guiding force in her work. Today, Sara blends evidence-based care with osteopathic principles of healing, working to support patients' mind, body and spirit. Her unique perspective as both a physician and sibling of someone with CF informs her dedication to advocacy, education, and meaningful connection in medicine.

Hema Patel lives in Southern California and is the mother of an amazing 34-year-old son living with cystic fibrosis, who was diagnosed at age 14. Hema also has another wonderful son, three years younger, who does not have CF and has been a strong source of support for his brother. Hema is an Associate Director for Global Business Travel in Worldwide Sales at Best Western Hotels, also known as BWH Hotel Group.

The CF diagnosis in 2003 changed the course of their lives and led Hema into a path of advocacy, support, and community engagement. Hema is actively involved with CFRI, serving on the Diversity, Equity, and Inclusion (DEI) Panel, the Gala Committee, and participates in advocacy efforts.

Hema also serves as a Peer Connect Mentor with the Cystic Fibrosis Foundation, supporting other parents as they navigate this complex and emotional journey.

Hema is with us today to share a mother's perspective of the CF journey, particularly around the challenges and impacts of a late diagnosis — to help amplify caregiver voices and advocate for a more inclusive, compassionate, and informed approach to CF care and support.

Gail Wright, RN, met her husband Bob, who lives with CF, at Camp Bothin, where a CF camp started by Dr. John Whalen of (then) Oakland Children's Hospital was held. They have been married for almost forty-one years and have three adult (adopted) children, one of whom lives with them and her three-year-old twins.

Gail grew up with a family with CF. Someone from the Cystic Fibrosis Foundation spoke at her nursing school, leading her to volunteer at CF camp, and lo these many years later, leading her to be part of this panel. She has worked in many nursing settings and is currently a Home Health nurse.

Fortunately, Bob manages his own health, and Gail is rarely his nurse – she gets to stay his wife instead! Like many in the CF community, they have seen other health issues besides CF.

Abstracts & Presenter Profiles

Guts, Glitter, Glory

Sunday, July 27, 11:00 am

Dylan Mortimer, MFA, *Long Beach, CA*

Dylan Mortimer was diagnosed with cystic fibrosis at three months old in 1979. He received a double lung transplant on January 18, 2017 and a second transplant on April 13, 2019. He and his wife Shannon were married in 2005 and have two boys who are now 16 and 13.

Dylan will share his artwork which reflects his journey through CF and lung transplant. The symbols in his work come from this experience: IV bags, scars, cells, bronchial trees etc. He transforms these objects in shiny material like glitter, which itself is dirty and spreads everywhere, much like disease itself. Yet it is beautiful, baroque and transformative. This transformation shifts all these symbols to joyful beacons of hope. And the excessive use of glitter symbolizes an aggressive hope and aggressive joy, the kind it takes to survive this kind of difficulty.

He has created public art installations in several cities including New York, Chicago, Baltimore, Kansas City, Sydney Australia, and Cape Town, South Africa. His exhibition history includes Columbia University in NYC, Casula Power House Museum in Sydney Australia, The Longwood Arts Gallery in the Bronx, the Dumbo Arts Center, David Zwiner Gallery in NYC, the Kansas City Jewish Museum, the Nerman Museum in Overland Park, KS, and the Haw Contemporary in Kansas City. He has been featured in the New York Times, The New York Post, The Chicago Sun, The Baltimore Sun, NPR, The Christian Science Monitor, The Kansas City Star, The Daily Mail, Sculpture Magazine, Public Art Review, and several other publications internationally.

Dylan will present his artwork and journey, and lead a creative activity, likely with messy glitter!

Dylan Mortimer, MFA, graduated with a BFA from Kansas City Art Institute and a MFA from the School of Visual Arts in New York. He has created public art installations in several cities including New York, Chicago, Baltimore, Kansas City and Denver. His exhibition history includes Columbia University, The Longwood Arts Gallery in the Bronx, the Dumbo Arts Center, PS 122 Gallery in New York, the Kansas City Jewish Museum, the Nerman Museum in Overland Park, KS, and the Haw Contemporary in Kansas City. He has been featured in the New York Times, The New York Post, The Chicago Sun, The Baltimore Sun, NPR, The Christian Science Monitor, The Kansas City Star, The Daily Mail, Sculpture Magazine, Public Art Review, and several other publications internationally.

2025 CFRI Awards and Awardees

Paul M. Quinton Cystic Fibrosis Research Legacy Award

This award is presented to a researcher who advances the understanding of cystic fibrosis, contributes to the search for a cure, inspires others to pursue CF research, has been funded by CFRI, and brings hope to the CF community.

Carol Conrad, MD (Posthumous Award)

Dr. Conrad, beloved pediatric pulmonologist at the CF Center at Lucile Packard Children's Hospital at Stanford, was the former director of the Pulmonary Function Lab and of the Lung and Heart-Lung Transplant Program at Stanford Medicine Children's Health. She received her medical degree from UCLA and trained in pediatrics at Children's Hospital Los Angeles. She completed fellowship training in pediatric pulmonary medicine at Johns Hopkins University and played a central role in demonstrating proof-of-concept for gene therapy in cystic fibrosis. Dr. Conrad and her team tested an approach using adeno-associated virus vectors to deliver the correct version of the gene. They found the delivered gene stayed active and kept working for up to six months, showing that it could be a promising tool for gene therapy to treat the disease. In 1995, Dr. Conrad arrived at Stanford Medicine, recruited by Dr. Rick Moss. She received CFRI funding in 1997 for a research project, "Efficacy of AAV-CFTR Vectors." Dr. Conrad was a beloved CF clinician who treated hundreds of pediatric CF patients during her 26+ years in clinic. In addition to serving as the director of the Pulmonary Function Lab, she served as medical director of Stanford Children's Pediatric Lung and Heart-lung Transplant Program for nearly 20 years. A brilliant researcher, Dr. Conrad studied lung inflammation that results from transplantation, including bronchiolitis obliterans, or chronic lung allograft dysfunction, a life-limiting condition that often affects lung and heart-lung transplant recipients. Dr. Conrad died unexpectedly in November 2024. She is remembered and missed by her family, colleagues and the countless patients and families who benefited from her exceptional care.

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

This award is presented to an adult with cystic fibrosis who has supported CFRI by volunteering with the organization and has displayed courage, initiative, determination, adherence to medical regimen, community service, and positive coping.

Anna Payne (Posthumous Award)

Anna Payne lived her life with energy and drive, and a commitment to uplift those around her. Her involvement with CFRI began in 2018, when she participated in CFRI's Externally-Led Patient Focused Drug Development meeting with the FDA. This led to her joining CFR's CF Adult Advisory Committee where she was a beloved member and actively involved with committee activities. In addition to her work with CFRI, Anna served on the Pennsylvania Rare Disease Advisory Council, served as an elected Township Supervisor, volunteered with the CFF, and worked for Bucks County Pennsylvania. In 2021, at the age of 34, just as she was celebrating a significant improvement in CF health due to Trikafta, Anna was diagnosed with stage-4 colon cancer. She faced this battle with bravery and pragmatism, and an unwavering commitment to help others avoid her fate. She pursued every means to raise awareness of the high incidence of colorectal cancer among those with CF, and the need for earlier screening. She wrote blog posts and participated in several CFRI podcasts and newsletter articles. She became an ambassador with Fight Colorectal Cancer and advocated in Washington DC. She formed the Bucks County CF Alliance, which raised funds to support CFRI's colon cancer advocacy efforts. During this time, she endured multiple surgeries and years of chemotherapy. She was a constant and vocal advocate for changes in the recommended age for first colonoscopy screening to help her peers with cystic fibrosis avoid a colon cancer diagnosis. Despite short periods of hope that she had achieved remission, Anna died from complications of colon cancer in February of this year. Her humor, quick wit, passion, and commitment to her CF community are greatly missed, but her legacy will continue.

2025 CFRI Awards and Awardees

CF Professional of the Year Award

This award is presented to an individual who has made an outstanding contribution in the field of cystic fibrosis through education, outreach, support, medical expertise, clinical practice or research.

Raksha Jain, MD, MSc

Dr. Jain is Medical Director of the Adult Cystic Fibrosis Program at UT Southwestern Medical Center and a Professor in the Department of Internal Medicine and a member of its Division of Pulmonary and Critical Care Medicine. Dr. Jain has been a leader in the field of pulmonology and has participated in research on an international scale that has translated to new therapies for those living with lung disease, particularly cystic fibrosis. Dr. Jain brings vision, hope, support and relief to those living with this devastating disease due to her extremely patient-focused clinical care, impactful research, and volunteer service. While Dr. Jain is a renowned expert in the area of cystic fibrosis and reproductive health, her research extends far beyond this topic. She has over 100 published research articles focusing on CFTR modulators, AAV mediated gene therapy, CF-related pancreatitis and autologous islet cell transplantation, sex-based difference in CF health outcomes, evaluation of inflammatory biomarkers, treatment strategies for resistant pathogens, mental health issues, lung transplantation, and bone health. This is just a small sampling! Dr. Jain's involvement with CFRI began many years ago, when she volunteered to present at CFRI's annual National CF Education Conference. She has since presented several times, and has participated in the creation of podcasts addressing CF and reproductive health. She shares her time sharing contacts with nationally-renowned leaders in the field of CF, who serve as speakers and panelists for CFRI's programs. Dr. Jain is committed to health equity and has advocated for a new roadmap for clinical trial recruitment on a global level.

Dave Stuckert Volunteer of the Year Award

This award is presented to a CFRI volunteer who has made outstanding contributions to the CFRI community through education, outreach and support.

Christine Nash, MBA

Christine Nash is recognized for her exceptional dedication and contributions to the CFRI community and unwavering commitment to advancing research and care for people with CF. Christine is the sister of the late Patrick Nash and the late Elizabeth Nash, both of whom lived with cystic fibrosis. Christine has worked in partnership with CFRI for many years through the Elizabeth Nash Foundation, which provides support for CFRI's research awards. In recent years, Christine has demonstrated exemplary service to the inaugural CFRI Patrick Nash Fellows Training Program, where she has played a vital role in shaping the program's vision and objectives to create a new model of multi-disciplinary care for adults with CF. Described as capable, smart, curious, kind, versatile, genuine, and a dynamo of masterful determination, Christine has helped to mobilize resources and inspire others. She has been instrumental in generating support for the Fellows Training program, which is in the midst of its second year. Christine's passion, empathy, and unwavering dedication have left an indelible mark on the CFRI community. As shared in the nomination, *"She has successfully directed the energy and grief of remembrance for those who have fought and lost their battle with CF into championing incredibly impactful programs...She is a shining example to the entire community."*

Special Thank You

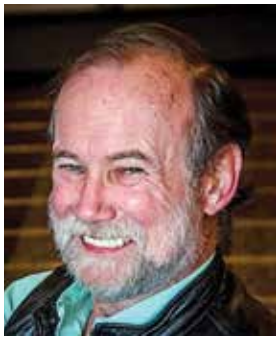
CFRI Professional and Volunteer of the Year Awards Panel

Zoe Davies, NP
Colleen Dunn, MS, RT, CCRD
Oscar Flamenco, CPA
Marina Gonzales
Alicia Maciel, MBA
Dennis Nielson, MD, PhD
Deepika Polineni, MD, MPH
Yelizaveta Sher, MD, FACLP
Kate Yablonsky, LCSW

CFRI Conference Committee

Sabine Breon, MA
Mary Convento
Barbara Curry
Hannah Dolhai, MSW
Leeya Kannankunni
Jennifer Keller, MD
Rohini Thukral McKee
Jane Mitchell, MSW
Stacie Reveles
Siri Vaeth, MSW

Awards Celebration Special Guests



Paul Quinton, PhD

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

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. Dylan is a realtor with Coldwell Banker in Santa Cruz County, California, focused on residential sales. Music is 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 once again DJ CFRI's annual dance party.

Support & Discussion Groups

CFRI's virtual Support and Discussion Groups offer an opportunity to gather with CF community peers to share experiences and information that are unique to those touched by cystic fibrosis.
The support groups are only available at the in-person event.

- *Adults with CF / Adults with CF post-transplant – Sonya Haggett, LCSW*
- *Parents / Caregivers of Children with CF – Deb Menet, LCSW*
- *Parents / Spouses/Partners/Siblings of Adults with CF – Kate Yablonsky, LCSW*

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

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

Support/Discussion Group Facilitators

Sonya Haggett, LCSW

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

Deborah Menet, LCSW

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

Kate Yablonsky, LCSW

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

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

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.

DONATE YOUR BIRTHDAY (OR OTHER SPECIAL EVENT) TO CFRI ON FACEBOOK — Setting up a birthday event on Facebook is free and easy. 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.

A BREATH OF FRESH AIR GALA — Saturday, October 11, 2025, in-person at the historic Hillsborough Racquet Club (Hillsborough, CA). Wine, dine, bid in our auction, and dance the night away. Sponsorships are available!

41st ANNUAL GOLD TOURNAMENT BENEFITTING CFRI — Held on Monday, August 18th, 2025 at the beautiful Cinnabar Hills Golf Club in San Jose. Golf with friends, wine and dine, and bid in both silent and live auctions

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.

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!

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.

PURPLE POWER CF AWARENESS CHALLENGE — During the month of May – Cystic Fibrosis Awareness Month – we invite you to share your favorite purple pics on social media, from purple outfits to purple food and furry friends (the possibilities are endless). Then tag CFRI, post on your page, and challenge others to join in, donate (or both).

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

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

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

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

CFRI Programs and Events

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

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

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

Monthly Online Support Groups for the CF Community

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

For Adults with CF — Third Monday of every month, 6:00 pm PT to 7:00 pm. An 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: Conocimiento y Conexión — 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 a CF social worker well versed in issues facing teenagers with CF. Parents must 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 an adult with CF and lung transplant recipient.

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

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

CFRI Programs and Events

Mindfulness Workshops

CFRI is offering quarterly workshops focused on different aspects of mindfulness and how it can offer positive coping skills for those living with CF. The workshops are led by Julie Desch, MD, certified mindfulness instructor and person living with CF. Next date: Tuesday, September 9 – Mindfulness to Cope with Sadness and Depression

CFRI's CFQoL Programs are generously supported by Viatris, Vertex Pharmaceuticals, Genentech, the 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, Viatris, and Genentech.

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 Viatris, AbbVie, Genentech, ReCode, and Gilead Sciences.

CF Spring and Summer Retreats

The annual CF Spring Retreat and CF Summer Retreat enhance education, positive coping skills, and social support for adults who share common experiences with CF, and include educational presentations, exercise, arts and crafts, support groups, and much more. The 2025 Summer Retreat will be held at the Jesuit Retreat Center in Los Altos, CA from August 14 – 19. ***Join us!***

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

CFRI Programs and Events

CF Wellness Initiative

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

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

Purple Power CF Awareness Challenge

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

Generously sponsored by Vertex Pharmaceuticals and Viatris.

Embrace Retreat for Mothers of Children and Adults with CF

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

Generously sponsored by AbbVie and Vertex Pharmaceuticals.

CF 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 Vertex Pharmaceuticals and Viatris.

CFRI Programs and Events

A Breath of Fresh Air Gala Event

On Saturday, October 11, 2025, join us for our annual gala and support the search for a CF cure. The in-person event will be held at the historic Hillsborough Racquet Club in Hillsborough, CA. In addition to inspiring stories, musical performances, gourmet food and local wines, we will honor our 2025 CFRI Champion, Dr. John Mark of Stanford. Bid in our exciting live and silent auctions and then dance the night away. A Breath of Fresh Air will support CFRI's research, education and support programs.

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

Patrick Nash Fellows Training Program – Aging in the New Era of CF

CFRI's Patrick Nash Fellows Training Program seeks to advance improved models of multidisciplinary care for adults with cystic fibrosis (CF) who face multiple complex health complications as they age (eg cancer, diabetes, liver desease, infertility). Named in honor of beloved CFRI community member Patrick Nash, who lived with CF and lost his life to pancreatic cancer, the program brings together practitioners from a wide range of specialties to expand their knowledge of CF adults' emerging multi-organ health complications, from a multidisciplinary cohort of providers, and explore collaborative research and multidisciplinary care models. The program educates and connects the next generation of care providers and thought leaders and serves as a catalyst for innovative research activities.

Generously sponsored by Vertex Pharmaceuticals, The Elizabeth Nash Foundation, and private donors.

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

CFRI Cystic Fibrosis Research Institute

RESEARCH · EDUCATION · ADVOCACY · SUPPORT

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

The Cystic Fibrosis Research Institute was founded in 1975 as an independent 501(C)3 nonprofit organization by a group of family members whose children had cystic fibrosis. Our mission is to be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support. Our vision is to find a cure for cystic fibrosis while enhancing quality of life for the CF community. We are able to provide our diverse programs and services thanks to our phenomenal volunteers, who generously share their time and expertise to advance research and improve the lives of those impacted by cystic fibrosis.