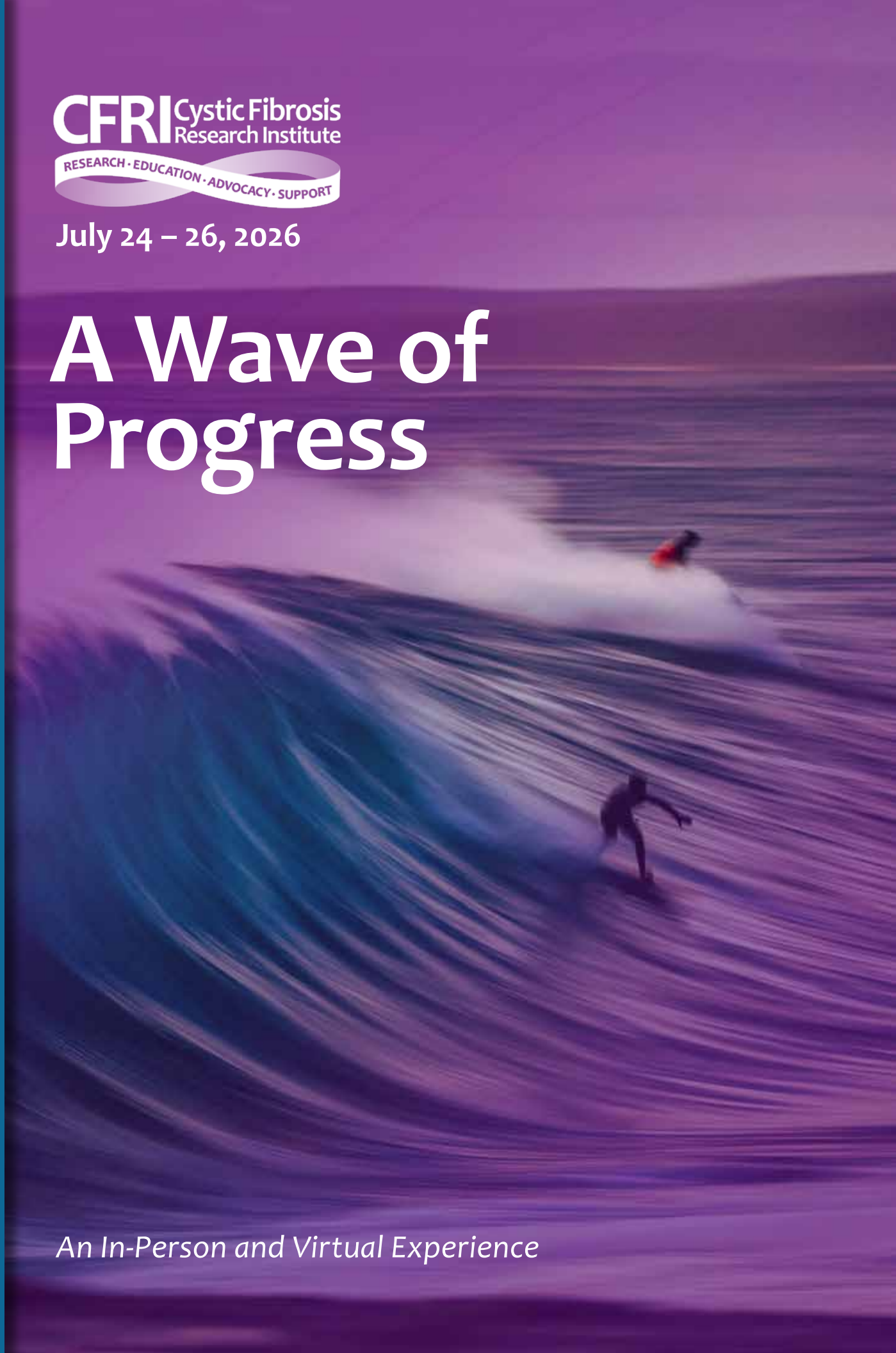
39th National Cystic Fibrosis Education Conference

CFRI Cystic Fibrosis Research Institute

RESEARCH · EDUCATION · ADVOCACY · SUPPORT

July 24 – 26, 2026

A Wave of Progress



An In-Person and Virtual Experience

Table of Contents

Welcome

Dear Friends,

Welcome to CFRI's 39th National Cystic Fibrosis Education Conference, A Wave of Progress. We are delighted that once again this year's conference is offered as a hybrid event, offering the opportunity for members of our community to come together in person and virtual, 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 to be made in the field of CF, and we are inspired and immensely proud of CFRI's role in these advances.

Our 2026 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 our phenomenal 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 2026 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.

CFRI is an organization by and for the CF community. Engage with us year-round; each of us plays a key role in advancing CFRI's goal to advance research while uplifting those impacted by this still-challenging disease.

Warm regards,

Julie Desch, MD
President, CFRI Board of Directors

Siri Vaeth, MSW
Executive Director, CFRI

Conference Schedule ~ *A Wave of Progress*

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

Friday, July 24, 2026

3:00 pm	**Registration Opens**
3:30 pm – 5:00 pm ***Pioneer*** ***Curiosity***	**Support Groups** (in-person only) — *Adults with CF Including Those Post-Transplant* — *Parents/Caregivers/Spouses/Relatives of Someone with CF*
5:00 pm – 6:45 pm ***Airstream***	**Reception and Welcome at the Airstream!**
7:00 pm – 7:45 pm ***Pioneer***	**Sleep and CF: Common Concerns and Intervention Targets in the CFTR Modulator Era** — Kimberly Canter, PhD *(Nemours Center for Healthcare Delivery Science)*
7:45 pm – 8:30 pm ***Pioneer***	**From Resistance to Resilience: Finding Balance on My CF Journey** — Niraj Patel

Saturday, July 25, 2026

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

8:00 am – 8:30 am ***Roger Restaurant***	**Breakfast**
8:45 am – 9:00 am ***Pioneer***	**Welcome and Opening Remarks** — Siri Vaeth, *MSW, CFRI Executive Director* / Rohini McKee, *Emcee*
Orion	— Devin Wakefield and Jean Hanley, MD, *Research Track Emcees*
9:00 am – 9:45 am ***Pioneer***	**CF Pharmaceutical Issues - Drug-Drug Interactions and Beyond** — Jake Brockmeyer, PharmD, BCPS, BCPPS *(Stanford Medicine Children's Health)*
Orion	**Pharmacogenomic Analyses of Corrector-Resistant Cystic Fibrosis** — Celeste Riepe, PhD *(Stanford University)*
9:55 am – 10:40 am ***Pioneer***	**Update on Nucleic Acid Therapies for Cystic Fibrosis: From Transient Replacement to Durable Genome Correction** — Manu Jain, MD *(Northwestern University)*
Orion	**Studying the Development and Function of FOXI1⁺ Pulmonary Ionocytes in Human iPSC-Derived Airway Epithelium** — Ruobing Wang, MD *(Boston Children's Hospital)*
10:55 am – 11:40 am ***Pioneer***	**From Carrier State to Overt CF - Impact on Risk of Cancer and Implications for Screening and Treatment** — Steven Freedman, MD, PhD *(Beth Israel Deaconess Medical Center)*
Orion	**Engineering Enhanced CFTR Variants to Advance Genetic Therapies for Cystic Fibrosis** — Brodie Ranzau, PhD *(Nationwide Children's Hospital)*
11:50 am – 12:35 pm ***Pioneer***	**Reproductive Health in Cystic Fibrosis: How CF Enriched my Life** — Sigrid Barrett, PhD, RN *(University of Nevada, Las Vegas School of Nursing)*
Orion	**Unraveling Ionocyte Heterogeneity and Responses to Cystic Fibrosis** — Feng Yuan, PhD *(University of Alabama at Birmingham)*
12:35 pm – 1:35 pm ***Infinity Foyer***	**Lunch** (Virtual: Exhibitor Hall / Lounge)

1:40 pm – 2:25 pm ***Pioneer***	**The Gut-Lung Axis in CF** — George O'Toole, PhD *(Dartmouth College)*
Orion	**CFTR Gene Repair by AAV-Mediated Base Editing in Cystic Fibrosis Pig Airway Epithelia** — Ashley Cooney, PhD *(University of Iowa)*
2:35 pm – 3:20 pm ***Pioneer***	**Cystic Fibrosis-Related Diabetes: Advancing Care for People with CF in an Evolving Landscape** — Kevin Scully, MB, BCh, BAO *(Brown University)*
Orion	**Optimization of Activity and Improved Delivery of Bacteriophages Targeting *Burkholderia spp*** — Daria Van Tyne, PhD *(University of Pittsburgh)*
3:35 pm – 4:20 pm ***Pioneer***	**CF in the Context of the Exposome: Recent Developments and Future Directions** — Gabriela Oates, PhD *(University of Alabama Birmingham)*
Orion	**Development of a Phage-Based Gene Delivery Platform for Restoring Wild-Type CFTR Expression on the Human Lung Epithelia** — Saumel Perez Rodriguez, PhD *(Stanford University)*
4:30 pm – 5:45 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 26, 2026

8:15 am – 9:00 am ***Roger Restaurant***	**Breakfast**
9:00 am – 9:05 am ***Pioneer***	**Welcome** — Siri Vaeth, *MSW, CFRI Executive Director*
9:05 am – 10:15 am ***Pioneer***	**Panel: Impacts of Aging with CF** — Art Brace, PhD; Julie Desch, MD; Laura Mentch, EdM / Moderated by Ahmet Uluer, DO, MPH *(Boston Children's Hospital/ Brigham and Women's* Hospital)
9:05 am – 9:50 am ***Orion***	**Towards Overcoming Evolved Bacteriophage Resistance via Next-Generation Directed Evolution** — Sage Dunham, PhD *(University of California Irvine)*
10:15 am – 10:30 am ***Pioneer***	**CFRI Programmatic Offerings** — Siri Vaeth, MSW, *CFRI Executive Director*
10:30 am – 11:00 am	**Break** — Room Check-Out
11:00 am – 11:45 am ***Pioneer***	**The Bloom Effect: How Resilience Transforms Us** — Kimberly Morse, MSW, LCSW *(Children's Hospital Los Angeles)*
Orion	**Factors that Promote Antibiotic-Tolerant *Staphylococcus aureus* CF Infections** — Daniel Wolter, PhD *(University of Washington Seattle)*
11:55 am – 12:40 pm ***Pioneer***	**The Stories We Inherit: CF, Community, and the Search for Awe** — Tré LaRosa *(Inspirational Presentation)*
Orion	**Designing a CF Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cells** — Ruby Sims, PhD *(University of California Los Angeles)*
12:40 pm – 12:45 pm ***Pioneer***	**Closing Remarks** — Siri Vaeth, *MSW, CFRI Executive Director*

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, 2026 by a CFF-accredited laboratory and must have a medical release signed by their CF medical team to attend.
- Please cover your mouth with a tissue when coughing and immediately disinfect your hands. Do not dispose of sputum in toilets or sinks.
- 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 39th National CF Education Conference

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Bronze Exhibitors — *Nestlé Health Science; Clarametyx Biosciences; 4DMT; Vytala*

Organizational Exhibitors — *Cystic Fibrosis Engagement Network (CFEN);*

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List current as of 07/07/2026. Updates to list available in digital program.

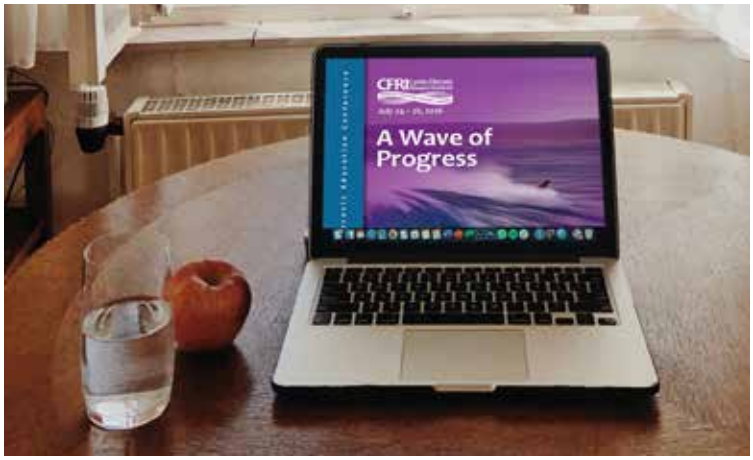
Tips for Navigating the Virtual Conference

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.

To access the conference you must first log in with your conference credentials. 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 or click on the ICON of the area you would like to access (Exhibit Hall – Theater – Agenda - Networking Lounge – Help Desk). If attendees miss any sessions they may view previous recordings.

Exhibit Hall

To visit a virtual booth, simply click on the Exhibit Hall and then the booth you are interested in viewing. There is a virtual scavenger hunt: the more booths you visit and sessions you attend, the higher your score, with a prize drawing at the end.

Theater

When you visit the virtual theatre, you will see the current session offerings. You can select from either the General or Research sessions. Having a hard time deciding which one to attend? All sessions are recorded and at their conclusion will be available for viewing. Need captioning or translation? You will see the option to have the presentation captioned in your language of choice.

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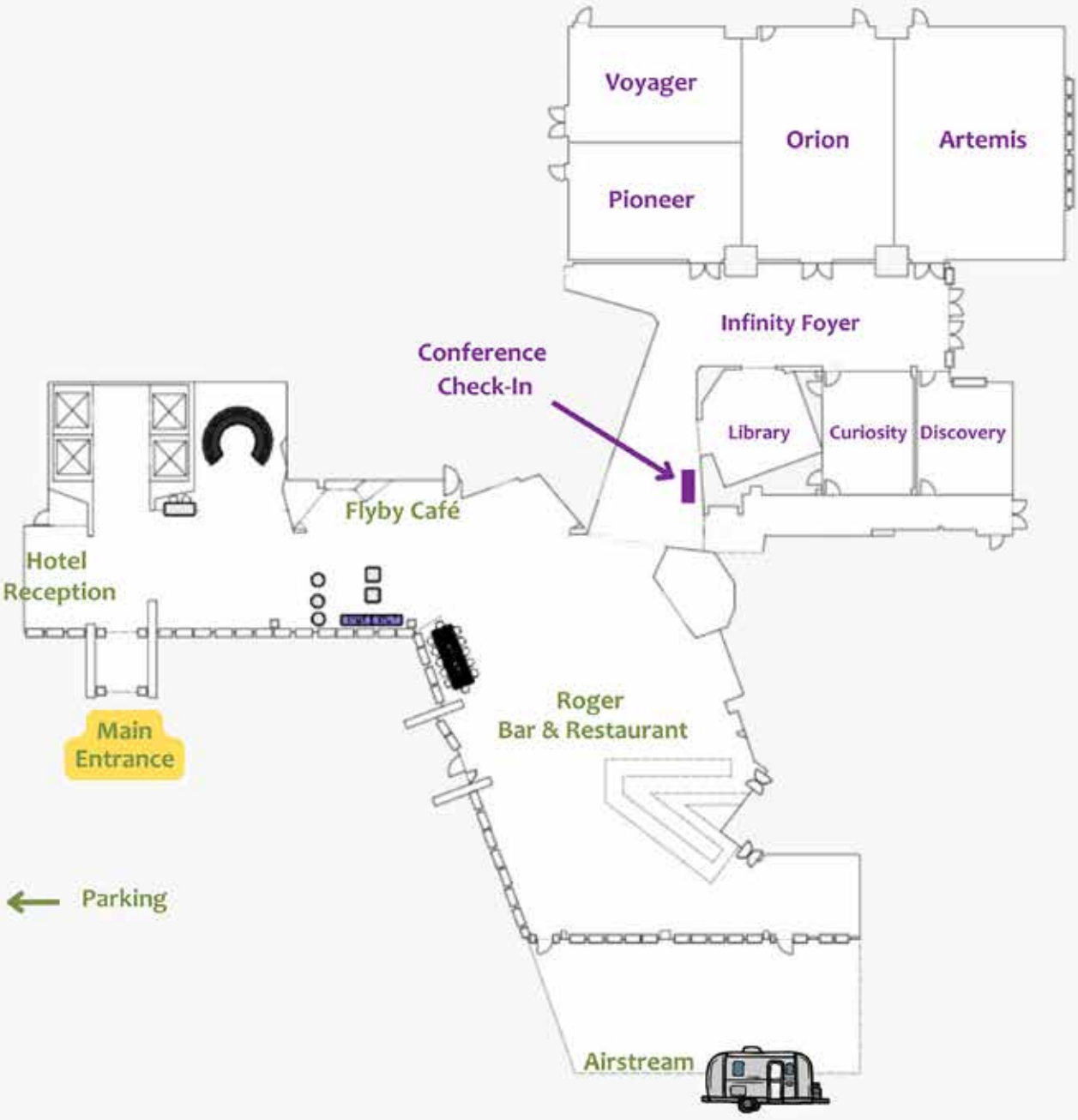


CFRI Board President, Executive Director and Emcees

Julie Desch, MD — CFRI Board President

Julie Desch serves as the President of the Board of Directors of CFRI and is a member of CFRI's Research Advisory Committee. At 65 years of age, Julie wakes up every morning amazed and grateful to be alive and healthy, breathing with her native lungs despite two copies of F508del. Her interest in CF research led her to Stanford Medical School, where she worked with Dr. Jeffrey Wine in the Cystic Fibrosis Research Laboratory as she pursued her medical degree. She then completed a residency and two fellowships in Anatomic Pathology at Stanford. After training, she worked at Kaiser Hospital in San Francisco, California as a surgical and skin pathologist. After retiring to take better care of herself and to be a full-time mom, she became a certified personal trainer and wellness coach for children and adults with CF before pursuing teacher training in Mindfulness-Based Stress Reduction, followed by a two-year Mindfulness Teacher Training Certification program, and then coach training in Unified Mindfulness. She has been teaching meditation online for the last ten years. She currently offers four workshops per year for the CF community, focusing on different aspects of mindfulness and how it can help people cope with the challenges of CF.

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 the proud Chair of the American Thoracic Society's Public Advisory Roundtable. Siri's daughter Tess is now 31, and her son Dylan is 27. She lives in Santa Cruz, California.

Rohini McKee — Emcee (General Session)

Rohini is the mother of two active and fun kids! Ria is 10 and living with cystic fibrosis and Rory is 4 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 Senior Director at Catalyst Exchange, an 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 CFRI's Board of Directors.

Continued on page 11

Continued from page 10

Devin Wakefield — Emcee (Research Track)

Devin is a 35-year-old man with cystic fibrosis. He currently is the Research Advisory Committee (RAC) co-chair. Devin also serves on CFRI's 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.

Jean Hanley, MD — Emcee (Research Track)

Dr. Jeanie Hanley is a physician living with cystic fibrosis. She has been married for 40 years and is mother to three adult children. Although she had CF symptoms since childhood, she was diagnosed in her early 30's only after genetic testing became available. Three of her nine siblings were also diagnosed with CF.

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

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

Abstracts and Presenter Profiles

** denotes CFRI-funded research*

Friday, July 24, 2026

Sleep and CF: Common Concerns and Intervention Targets in the CFTR Modulator Era

Friday, July 24, 7:00 pm

Kimberly Canter, PhD, *Nemours Center for Healthcare Delivery Science / Wilmington, DE*

Sleep concerns, including insomnia, are extremely common among people with CF. Sleep concerns are frequently associated with worse physical health outcomes and are often comorbid with mental health concerns, such as depression. Moreover, while CF transmembrane conductance regulator modulator therapies (CFTRm) have improved health outcomes for many people with CF, sleep concerns have persisted and even worsened for some post-CFTRm initiation. This presentation will summarize the literature describing sleep disturbances and disorders that impact people with CF, including emerging information about the possible impact of CFTR dysfunction on circadian rhythms. Information regarding the effects of CFTRm therapy on sleep in pediatric and adult populations will also be reviewed. The presentation will conclude with a discussion about evidence-based treatment options for people with CF who are experiencing sleep concerns.

Kimberly Canter, PhD, is a Pediatric Psychologist, Senior Research Scientist at the Nemours Center for Healthcare Delivery Science, and Associate Professor of Pediatrics at Thomas Jefferson University. She is also Core Director of the Intervention Methodology: Provision and Connection through Technology (IMPACT) Research Core of the Research Expanding Access to Child Health (REACH) Center, a Center of Biomedical Research Excellence (COBRE) based at Nemours Children's Health in Delaware. Her research focuses broadly on developing and implementing evidence-based, technology-enabled psychosocial interventions to improve health, decrease traumatic medical stress, and enhance coping skills for people with chronic illnesses and their families. She has worked extensively in cystic fibrosis and serves as co-PI for Nemours Children's Health in the Success with Therapies Research Consortium, which is a multisite research network that develops and tests interventions to improve disease self-management and strategies to disseminate and sustain interventions for children and adults in real-world settings. Dr. Canter has a specific interest in interventions that are wide-reaching and helpful in terms of increasing access and reducing barriers to care, as well as in partnering with community members and stakeholders to conduct and disseminate research. Her work has been funded by the Cystic Fibrosis Foundation, American Cancer Society, Alex's Lemonade Stand Foundation, the National Cancer Institute, and the National Institute of General Medical Sciences.

From Resistance to Resilience: Finding Balance on My CF Journey

Friday, July 24, 7:45 pm

Niraj Patel, *Frisco, TX*

I don't generally talk about myself and CF. I spent most of my life fighting to let it be a part of my identity. I was diagnosed at 14, and telling a troubled teenager who rebelled against everything that his life will fundamentally change must have been a nightmare for everyone involved. I fought for a long time to distance myself as far as I could from everything to do with CF.

Transitioning into early adulthood, this rebellion was mainly for the worse. Making bad decisions that were outright detrimental to my wellbeing, I actively ignored the fact that I had CF and disregarded treatment entirely. I'm not a great patient; I reschedule appointments and forget to take meds. This attempt to distance myself caught up with me, and I landed myself in the hospital during the lowest point in my life. A dead-end $12/hour job with no future, and a medical condition that only reared its ugly head during the most difficult times.

That sense of aloneness was the hardest feeling I've ever had to work through. I spent so long shutting out this condition that I didn't know what I would do. But the thing about people that truly love you, is that their love is unconditional. It wasn't any sort of internally found motivation that helped me back up; it was the love that I found from everyone else around me that helped me realize that I was never alone.

This presentation will journey through my life, diagnosis, how fighting my diagnosis impacted me, and how the people around me continue to help me find that balance. I would not be where I am if it weren't for those people.

Niraj Patel is a software engineer who works for a bank. He loves what he does and loves to learn new things about it. His favorite hobbies are mainly nerd-based: Video games (he plays a lot of video games), technology (he loved building his PC and would love to do another build someday when prices are better, working on his home server, etc.), keeping up with the world, sports (basketball and soccer mostly), food (he loves exploring new cuisines), learning and reading about something new. When he is not doing those things at home, he is hopefully traveling somewhere, which is his favorite thing to do. He has been to a few cool countries and would like to explore as much of the world as he can. Just recently he traveled to Italy and Japan and is hoping to go to India and China next year. His favorite place to go is the UK, since he was born there and it still feels like home. But after visiting Japan, it has definitely made itself a contender for favorite.

Niraj was diagnosed with CF at 14 and had a harrowing journey to diagnosis. Being a patient with CF has its drawbacks but he enjoys sharing his diagnosis story in hopes that it spreads awareness for the often difficult impact of race in CF diagnosis and care, as the failure to diagnose him earlier was based on that.

Favorite motto to live by: If you think you have learned enough in this world, then you aren't doing life correctly. There is endless knowledge in this world, and one can never learn everything. That is the beauty of life, there is always more to learn.

Saturday, July 25, 2026

CF Pharmaceutical Issues – Drug-Drug Interactions and Beyond

Saturday, July 25, 9:00 am

Jake Brockmeyer, PharmD, BCPS, BCPPS, *Stanford Medicine Children's Health / Palo Alto, CA*

Cystic fibrosis pharmacotherapy has become more effective but not simpler. Although highly effective CFTR modulators have transformed outcomes for many patients, most people with cystic fibrosis still require complex multidrug regimens that include pancreatic enzyme replacement therapy, fat-soluble vitamin supplementation, airway clearance medications, inhaled antibiotics, and recurrent antimicrobial exposure. This presentation highlights a pharmacist-forward approach to medication management in cystic fibrosis, emphasizing practical issues that affect both families and clinicians. Key topics include clinically important modulator drug-drug interactions mediated by CYP3A4, especially with azole antifungals, ritonavir-containing regimens, and rifamycins; continued optimization of pancreatic enzymes and vitamins in the modulator era; treatment sequencing for bronchodilators, mucolytics, airway clearance, and inhaled antibiotics; and medication access barriers requiring specialty pharmacy and copay support coordination. The session also addresses emerging mental health concerns related to CFTR modulators, including the March 2026 label update adding a warning for neuropsychiatric events, including suicidal thoughts and behaviors. Using current peer-reviewed literature and pharmacist-relevant cases, this talk frames the cystic fibrosis pharmacist as central to medication counseling, toxicity monitoring, adherence support, interaction management, and access continuity. Attendees will leave with practical strategies to recognize high-risk interactions, improve day-to-day counseling around enzymes, vitamins, and inhaled therapies, and integrate mental health screening and escalation into modulator care without discouraging use of these highly beneficial therapies.

Jake Brockmeyer, PharmD, BCPS, BCPPS, is a double board-certified, Pediatric Clinical Pharmacist who completed both a general PGY-1 and pediatrics specific PGY-2 residency at Lucile Packard Children's Hospital at Stanford. He earned his Doctor of Pharmacy from Regis University School of Pharmacy in Denver, Colorado. He is experienced in a wide range of medically complex disease states. His professional interests include cystic fibrosis, infectious disease, pharmacokinetics, therapeutic drug monitoring, and precision medicine.

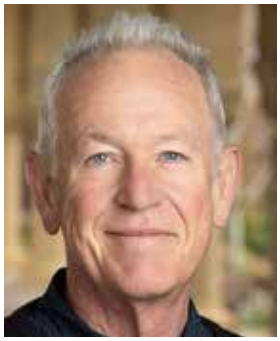
* Pharmacogenomic Analyses of Corrector-Resistant Cystic Fibrosis

Saturday, July 25, 9:00 am

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

Small-molecule correctors that promote the folding of CFTR have become the standard treatment for people with cystic fibrosis (pwCF), yet thousands are not helped by correctors, either because they exhibit adverse side effects or because they express CFTR variants that do not respond to the drugs. The lack of effective, mechanism-based therapies for pwCF who produce full length CFTR yet cannot take correctors is a critical unmet need. Development of new treatments is hampered by a profound gap in knowledge of the cellular protein quality control (PQC) machinery that promotes CFTR trafficking and degrades CFTR in the endoplasmic reticulum (ER) and post-ER compartments. Here, we exploit CRISPR-based pharmacogenomic analyses to fill this gap by identifying the PQC machinery that underlies triage of corrector-sensitive F508del and corrector-resistant W57G, L227R, I507del, W1098R, and N1303K variants. Firstly, while correctors facilitate F508del trafficking to the plasma membrane, F508del is less stable than WT and is degraded through a noncanonical cholesterol-dependent, dynamin-independent endolysosomal pathway. Secondly, resistant variants either 1) failed to escape ER-associated PQC, or 2) escaped ER-PQC but failed to exit the ER in the presence of correctors. Genome-wide CRISPR screens with corrected N1303K revealed that failure to exit the ER could not be restored by inhibiting cellular machinery. Lastly, the trafficking and function of W1098R could be fully restored with the preclinical corrector, IDOR-4. Taken together, we have identified strategies orthogonal to FDA-approved correctors for restoring CFTR trafficking and function that can be used to design the next-generation of CF therapeutics.

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 some people with cystic fibrosis fail to respond to modulator therapies. A native of San Marcos, TX, Dr. Riepe received a BA 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. 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.



Ron Kopito, PhD, 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.

Update on Nucleic Acid Therapies for Cystic Fibrosis: From Transient Replacement to Durable Genome Correction

Saturday, July 25, 9:55 am

Manu Jain, MD, *Northwestern University / Chicago, IL*

Cystic fibrosis is a compelling target for nucleic acid therapy because it is caused by pathogenic variants in a single gene, CFTR, yet important therapeutic gaps remain despite the transformative impact of CFTR modulators. Individuals with non–protein-producing mutations, rare variants, incomplete responses, or intolerance to modulators continue to need approaches that address the underlying genetic defect more broadly. This lecture will review recent nucleic acid-based trials and programs for cystic fibrosis, including mRNA replacement, gene replacement and short nucleotide approaches. These platforms differ in durability, complexity, and risk: mRNA replacement offers a mutation-agnostic, non-integrating strategy but will likely require repeat dosing; gene replacement may provide longer CFTR expression but faces vector and redosing challenges and short nucleotide approaches tend to be genotype specific.

Also discussed will be the challenges specific to the lung as a therapeutic target, including cellular heterogeneity, mucociliary barriers, inflammation and epithelial turnover. Recent insights into airway epithelial heterogeneity, including the roles of secretory cells, ionocytes, and basal progenitor cells, will be used to frame the central translational question: which cells must be corrected, and for how long, to produce meaningful clinical benefit? Gene editing and writing offer the possibility of durable correction but require precise and safe delivery to the appropriate airway cells. The final section will place cystic fibrosis in the broader therapeutic landscape by reviewing successful and emerging non-CF examples of mRNA therapeutics, approved gene replacement therapies, and clinically validated genome editing, highlighting how these advances inform the next generation of CF therapies.

Manu Jain, MD, is a Professor of Medicine and Pediatrics at Northwestern University's Feinberg School of Medicine. He graduated from Northwestern University with a Bachelor of Arts degree in Biochemistry and received his MD from The University of Chicago where he also completed his residency in Internal Medicine and fellowship in Pulmonary Critical Care. He joined the faculty at Northwestern in 1996 and has risen through the ranks as a full professor. Dr. Jain has been the director of the adult CF program at Northwestern since 1998. He has received intramural funding from the NIH, Veteran's Administration and CF Foundation for his investigator-initiated research. He has published more than 100 peer-reviewed articles and has been on numerous national CF boards and committees. He served as the adult program representative on the CF Foundation's planning committee for the North American Cystic Fibrosis Conference for 10 years. In addition, he has been on the Therapeutics Development Network (TDN) steering committee and has been the past chairman of the TDN protocol review and co-chair of the Guidelines Steering Committee. He is presently co-chair of the Genetic Therapies Working Group for CFF. He is on the editorial board for the AJRCCM and has been a reviewer for JCI, CHEST, Thorax, EMBO molecular medicine, Journal of CF, Translational Research among other journals. He has also served as an ad-hoc reviewer for numerous NIH panels, CFF, VA administration and CF Canada.

* Studying the Development and Function of FOXI1⁺ Pulmonary Ionocytes in Human iPSC-Derived Airway Epithelium

Saturday, July 25, 9:55 am

Ruobing Wang, MD,* *Boston Children's Hospital / Boston, MA*

Rationale: Pulmonary ionocytes are a rare airway epithelial population enriched for CFTR expression, yet studies across species and human systems have suggested diverse roles in airway ion transport and hydration. Human induced pluripotent stem cell (iPSC)-derived airways provide a tractable platform for precise genetic manipulation to define ionocyte function and uncover the developmental pathways governing their specification.

Results: To investigate ionocyte function, we generated syngeneic FOXI1 wild-type (WT), FOXI1-knockout (KO), and doxycycline-inducible FOXI1-overexpressing (OE) iPSC airway models using CRISPR-Cas9 engineering. FOXI1 deletion abolished ionocyte differentiation, altered epithelial composition with increased mucus and secretory cells, and significantly reduced forskolin-stimulated, CFTR-dependent chloride transport. Induction of FOXI1 restored ionocyte markers and rescued CFTR-mediated transport in the FOXI1-KO background. Incorporation of WT ionocyte-enriched basal cells (5%) into F508del homozygous CF basal cells (95%) partially restored CFTR-dependent chloride transport, with appreciable rescue observed at ionocyte frequencies as low as 1–2% and detectable activity at subphysiologic levels (0.25–0.5%).

To define mechanisms governing ionocyte development, temporal modulation of NOTCH signaling revealed biphasic regulation of ionocyte differentiation: early NOTCH inhibition suppressed ionocyte formation, whereas late inhibition markedly expanded FOXI1-, CFTR-, and BSND-associated programs through an active protein synthesis-dependent mechanism. Transcriptomic analyses identified candidate pathways including SHH, Wnt, and HIF2α signaling. SHH activation synergized with late NOTCH inhibition to enhance FOXI1 expression, supporting a combinatorial model of ionocyte specification. We further generated FOXI1- and BSND-based fluorescent reporter and inducible-ablation iPSC lines, enabling visualization and selective deletion of immature and mature ionocyte populations.

Conclusions: These studies establish a human iPSC platform to interrogate pulmonary ionocyte development and function. We demonstrate that FOXI1⁺ ionocytes are critical regulators of CFTR-mediated chloride transport and identify developmental pathways that may be leveraged to manipulate these cells therapeutically. Together, these versatile model systems provide a framework to advance airway cell biology and accelerate the development of future cell-based and mutation-agnostic therapies for CF and other respiratory diseases.

Dr. Ruby Wang's research program investigates the cellular and molecular mechanisms underlying pediatric airway diseases, with a focus on cystic fibrosis (CF), using human stem cell–derived models and gene-editing approaches. She integrates stem cell biology, molecular genetics, ion transport physiology, computational analysis, and host–pathogen biology to define how epithelial cell identity and function contribute to disease pathogenesis and therapeutic response.

During her clinical training in pediatric pulmonology at Boston Children's Hospital, Dr. Wang cared for children with severe CF lung disease whose phenotypes were not fully explained by their primary CFTR mutations. To address this gap, she established a cohort of patients with extreme CF phenotypes and generated patient-specific induced pluripotent stem cell (iPSC) lines. In collaboration with the Harvard Stem Cell Institute, she created syngeneic gene-corrected lines using CRISPR-based editing, enabling rigorous analysis of variant-specific effects in controlled genetic backgrounds. She subsequently trained in iPSC-directed airway differentiation at Boston University, and established stem cell–derived airway epithelial models in her own laboratory to study epithelial ion transport and CFTR dysfunction.

A central focus of Dr. Wang's work is the pulmonary ionocyte, a rare airway epithelial cell type enriched for CFTR expression and essential for airway ion homeostasis. She was the first to demonstrate de novo generation of pulmonary ionocytes from human iPSCs and developed reporter and gene-edited lines

to enable lineage tracing and functional perturbation of ionocyte-specific programs. These platforms allow cell-type–resolved analysis of CFTR function and epithelial ion transport. She also applies gene-edited iPSC-airway and primary airway models to study viral and bacterial infections in diseased airway epithelium, examining how epithelial composition affects host defense and treatment response.

From Carrier State to Overt CF – Impact on Risk of Cancer and Implications for Screening and Treatment

Saturday, July 25, 10:55 am

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

CF carriers, like people with overt CF, are at risk for inflammatory conditions such as pancreatitis, sinusitis, and liver disease. Recent data indicate that cancer risk is also elevated in both populations, suggesting that even partial CFTR dysfunction, as seen in carriers, contributes to the risk of developing cancer.

Overt CF is associated with a 7-fold increased risk of colorectal cancer, a 3-5 fold increased risk of pancreatic cancer, and elevated rates of breast and lung cancer. One published study suggests that CF carriers are at intermediate risk, pointing to a CFTR "dosing" effect on cancer development. Consistent with this, preliminary data from our group using a CF mouse model demonstrate pancreatic and colon cancer growth is markedly increased in CF mice compared with normal littermates, with intermediate growth rates in CF carrier mice.

The key question is whether we can predict who is at risk and prevent these cancers. Data from our NICE-CF study show that noninvasive stool marker testing is not predictive of lesions detected on colonoscopy. Moreover, the risk of developing adenomatous colon polyps or cancer was not decreased in patients receiving CFTR modulators.

How should these findings shape screening and treatment? For CF carriers, active studies are being initiated to confirm if there is an elevated cancer risk; if confirmed, colonoscopy and pancreatic MRI screening strategies should be implemented. For people with CF, screening protocols beyond colonoscopy, including for pancreatic cancer, must be defined with potential blood test markers, alongside preventive strategies such as dietary and other interventions.

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 at 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.

* Engineering Enhanced CFTR Variants to Advance Genetic Therapies for Cystic Fibrosis

Saturday, July 25, 10:55 am

Brodie Ranzau, PhD,* *Nationwide Children's Hospital / Columbus, OH*
Sriram Vaidyanathan, PhD,* *Nationwide Children's Hospital / Columbus, OH*

Approximately 10-50% of people with cystic fibrosis (pwCF) from various ethnicities have genotypes that do not respond to modulator therapies, highlighting the need for a durable gene therapy for CF that restores cystic fibrosis transmembrane conductance regulator (CFTR) function in airway stem cells. Our long-term goal is to develop a genome editing-based therapy for all pwCF in which functional CFTR cDNA is inserted into the CFTR locus of airway basal cells. We previously showed that this approach restores median non-CF

levels of CFTR function in vitro when 50-80% of cells are corrected. However, both in vivo gene insertion of basal cells and transplantation of ex vivo-edited basal cells remain inefficient. We hypothesized that delivering an enhanced CFTR variant with increased ion transport activity could lower the editing threshold for functional restoration and facilitate the clinical translation of gene therapies for CF.

We examined the functional performance of 10 different CFTR variants reported to increase protein stability or ion channel open probability, evaluating whether they restored CFTR function more effectively than wild-type (WT) CFTR. In airway basal cells from 4-5 CF donors, Cas9/HDR-mediated insertion of the ΔRI, I539T, or G550E CFTR variants displayed CFTRinh172-sensitive chloride currents of: 13.21 ± 7.24 μA/cm2, 17.23 ± 8.09 μA/cm2, and 12.74 ± 6.23 μA/cm2, respectively; higher than WT-CFTR corrected CF-cells: 6.35 ± 3.60 μA/cm2. This enhancement of function enables the restoration of median non-CF levels of function with 2-3 fold fewer corrected cells than with WT-CFTR, facilitating the development of durable genetic therapies for CF.

Brodie Ranzau, PhD, is a Postdoctoral Scientist in the Center for Gene Therapy at Nationwide Children's Hospital in Columbus, Ohio, where he works under the mentorship of Dr. Sriram Vaidyanathan. His research focuses on protein engineering and developing gene-based therapeutics for genetic diseases, with a particular emphasis on cystic fibrosis.

He earned his PhD from the University of California, San Diego, in the laboratory of Dr. Alexis Komor, where he developed expertise in protein engineering and precision genome editing technologies. This training sparked his interest in using protein engineering to overcome limitations in genetic therapies and enable durable treatments for people with genetic diseases.

As an Elizabeth Nash Memorial Fellowship awardee, Dr. Ranzau is engineering enhanced variants of the CFTR protein to support the development of durable, mutation-agnostic genetic therapies for all people with cystic fibrosis. His work has identified several CFTR variants with improved function, offering a promising path toward strengthening the performance of emerging cystic fibrosis gene therapies and enabling better outcomes with lower treatment requirements.

Sriram Vaidyanathan, PhD, is a Principal Investigator in the Center for Gene Therapy at the Abigail Wexner Research Institute at Nationwide Children's Hospital and an Assistant Professor of Pediatrics at The Ohio State University College of Medicine. His laboratory focuses on developing genome-edited stem cell therapies for airway disorders, with a primary focus on cystic fibrosis (CF). This includes efforts to improve the insertion of large DNA sequences using CRISPR-Cas9 and to develop methods for transplanting stem cells into the airways. He earned his Bachelor's degree in Biomedical Engineering from Purdue University and his PhD in Biomedical Engineering from the University of Michigan, Ann Arbor. He completed his postdoctoral training with Dr. Matthew Porteus at Stanford University.

Reproductive Health in Cystic Fibrosis: How CF Enriched my Life *Saturday, July 25, 11:50 am*

Sigrid Barrett, PhD, RN, *University of Nevada, Las Vegas School of Nursing / Las Vegas, NV*

This presentation examines the complex intersection of cystic fibrosis (CF) and reproductive health, utilizing an integrative framework that merges decades of advanced clinical expertise with profound lived experience. Grounded in a 28-year professional legacy focused on optimizing CF outcomes and advancing family-centered care models, the session leverages unique, first-hand insights into the arduous path required to achieve reproductive goals in this population. A central objective is shifting the traditional paradigm of CF management, a transition driven by revolutionary therapeutic advancements. By integrating clinical rigor with the imperatives of reproductive health advocacy, this session equips researchers, clinicians, and CF community members with a forward-looking framework to address evolving holistic needs. The presentation concludes with a strategic call to action for dedicated, translational research, reinforcing the collective mission to advance care and outcomes until a cure is found.

Sigrid Barrett, PhD, RN, PNP, CNE, FAAN, joined the University of Nevada Las Vegas (UNLV) in September 2024 as Dean for the School of Nursing. Barrett is a certified nurse educator and nurse practitioner specializing in pediatric acute and chronic care.

Prior to joining UNLV, Barrett spent ten years at the University of Alabama at Birmingham (UAB) School of Nursing. As a tenured Professor, she served as the Chair of the Department of Family, Community and Health Systems and contributed to several university-wide interdisciplinary research centers, including the UAB Gregory Fleming James Cystic Fibrosis Research Center, Center for Palliative and Supportive Care, and Minority Health and Health Disparities Research Center. Her program of research in cystic fibrosis, reproductive health, and quality of life in adolescents and adults with chronic illness have been supported by extramural funding from the National Institutes of Health, Cystic Fibrosis Foundation, etc. She has a strong track record of scholarship with nearly 100 peer-reviewed publications and 300 presentations across local, regional, national and international venues.

Her leadership in nursing education is reflected in her involvement with the National League for Nursing and the Philippine Nurses Association of America, where she has held several leadership positions at the national and local levels. For her impactful work in policy, research, education, and service, Barrett was selected as a Fellow of the American Academy of Nursing in 2020. She has also received awards for her excellence in research, teaching, and mentorship.

Barrett received her Bachelor of Science in Nursing at the University of Pennsylvania. She returned to the University of Pennsylvania for her Master of Science in Nursing, specializing as a pediatric acute/chronic nurse practitioner and clinical nurse specialist. In 2013, Barrett earned her Ph.D. in Nursing from the University of Central Florida.

* Unraveling Ionocyte Heterogeneity and Responses to Cystic Fibrosis

Saturday, July 25, 11:50 am

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

Cystic fibrosis (CF) is a genetic disease caused by mutations in the CFTR gene. Ionocytes are a newly identified epithelial cell type in the airway epithelium that expresses high levels of CFTR. Using single-cell RNA-seq (scRNA-seq), we characterized ionocyte heterogeneity and compared conditions with normal and ionocyte-specific CFTR deletion to assess the transcriptional programs of ionocyte differentiation. In vivo scRNA-seq of the nasal respiratory epithelium revealed that ionocytes account for 16.7% of epithelial cells. We also observed a higher prevalence of ionocytes in the nasal epithelium of CF compared to WT ferrets. We developed a single-cell transcriptomic landscape of ionocytes, comprising 398 ionocytes from in vivo and 7,650 from in vitro samples. Ionocytes obtained in vivo exhibited transcriptome profiles comparable to those observed in vitro, including key genes such as CFTR, BSND, and SLC12A2. Our transcriptional analysis revealed that ionocytes constitute markedly heterogeneous populations, with three subtypes defined by distinct transcriptional programs. The deletion of CFTR in ionocytes led to transcriptional reprogramming in airway epithelial cells. For example, under ionocyte-specific CFTR deletion, LEF1, a recently identified regulator of ionocyte fate specification, together with ATP6V0D2 and ATP6V1G3, subunits of the vacuolar H^+-ATPase involved in proton transport, were up-regulated in ionocytes, whereas secretory cells exhibited increased expression of AQP3 and SCNN1G. Future work will focus on characterizing ionocyte subtype-specific functions and the signaling pathways that modulate ionocyte fate.

Feng Yuan, PhD, is an Assistant Professor at the School of Medicine, Division of Pulmonary, Allergy, and Critical Care Medicine at the University of Alabama at Birmingham. He completed his training under Dr. John Engelhardt at the University of Iowa. He has received a Cystic Fibrosis Research Institute New Horizon Award and a Cystic Fibrosis Foundation Research Grant. His work demonstrated that pulmonary ionocytes are responsible for airway surface

liquid absorption and secretion through CFTR-dependent chloride transport. He is especially interested in developing strategies to fix defective transepithelial chloride transport in cystic fibrosis, which could help all cystic fibrosis patients.

The Gut-Lung Axis in CF

Saturday, July 25, 1:40 pm

George O'Toole, PhD, *Dartmouth College / Hanover, NH*

Clinical studies from our team and others show early-life dysbiosis in the intestinal microflora for children with CF (cwCF). This dysbiosis includes depletion of the key immune-modulating microbe Bacteroides spp. and increase in Proteobacteria (particularly *Escherichia coli*) for cwCF 0–1 years of age, and persists during the key immune programming window of 0–3 years of age, with recent findings from Dartmouth showing that cwCF have a microbiota which is delayed in the typical maturation process. Importantly, this gut dysbiosis is linked with worse clinical outcomes in airway disease. The mechanism(s) driving this intestinal dysbiosis have not been elucidated. Using a recently developed CF-relevant gut medium called CF-MiPro we were able to recapitulate the depletion of *Bacteroides spp*. and increase in E. coli in vitro, and furthermore, we showed that this competitive disadvantage was driven by the increased fat levels associated with the CF gut. Our studies show that two key factors appear to drive E. coli's competitive advantage in a CF gut-like environment: (i) the ability to catabolize the fat associated with the CF gut and (ii) the production of colibactin, a toxin that is associated with an increased rate of DNA damage and colorectal cancer. We have also evolved *Bacteroides spp* capable of growth in the CF gut environment. These findings will allow us to develop methods to correct the gut dysbiosis in CF to mitigate airway disease, while also understanding mechanisms that drive microbial competition.

George O'Toole, PhD, joined the Geisel School of Medicine as an Assistant Professor in 1999 and was promoted to Professor in 2010. He received his PhD from the University of Wisconsin-Madison with Dr. Jorge Escalante-Semerena and performed postdoctoral studies at Harvard Medical School as a Damon-Runyon and a Hood Fellow with Dr. Roberto Kolter. His honors include the NSF Career Award, Dupont Young Investigator Award and Pew Scholar in the Biomedical Sciences, election as a fellow of AAAS and the American Academy of Microbiology, serving as an Editor for the Journal of Bacteriology and receiving the 2025 ASM Graduate Education Award. Dr. O'Toole has worked in bacterial systems for ~20 years. His lab has published extensively in the area of P. aeruginosa-host interactions in the context of CF, including polymicrobial airway infections, as well as studying the gut-lung axis in CF. His lab also studies early bacterial biofilm formation by pseudomonads, with a focus on surface sensing and c-di-GMP signaling networks.

* CFTR Gene Repair by AAV-Mediated Base Editing in Cystic Fibrosis Pig Airway Epithelia

Saturday, July 25, 1:40 pm

Ashley Cooney, PhD,* *University of Iowa / Iowa City, IA*

CFTR modulators have transformed care for people with CF (pwCF); however, ~10-20% of pwCF harbor non-druggable mutations or are intolerant of modulators, underscoring the need for alternative therapeutic strategies. Recent advances in adeno-associated virus (AAV) vectors and gene editing technologies offer a path toward permanent correction of point mutations at the endogenous CFTR genomic locus. While our long-term goal is to target nonsense mutations such as R553X and W1282X, we first focused on correcting the proof-of-principle G551D mutation in primary CFTRG551D/G551D pig airway epithelial cells (PAE) cultured at air–liquid interface and benchmarking functional recovery against ivacaftor. We engineered a library of AAV capsid variants through a barcoded enrichment platform performed on primary human non-CF and CF airway epithelial cells. From this screen, we identified the AAVB5 capsid, achieving >75% transduction efficiency in PAE. Subsequently, we delivered AAVB5 carrying a cassetteencoding an adenine base editor (ABE) to mediate A□G conversion to edit the mutant aspartic acid to glycine residue. Specifically, we used the S. aureus Cas9 KKH variant that expands PAM compatibility, enables flexible editor positioning, and permits packaging in a single AAV vector. AAVB5-ABEpG551D vectors achieved efficient correction of the pig G551D mutation in vitro, resulting in

restoration of CFTR channel activity to levels comparable to ivacaftor, with minimal bystander editing. This work represents a pivotal step toward a durable gene therapy approach for CF and highlights the potential of a compact ABE delivered by a novel airway-tropic AAV capsid to achieve pulmonary gene editing.

Ashley Cooney, PhD, is an Assistant Professor at the University of Iowa developing innovative therapeutics for cystic fibrosis. Her laboratory focuses on gene therapy, including both gene addition and gene editing strategies. Specifically, using adeno-associated virus (AAV) to deliver adenine base editors targeted to restore the CFTR-G551D mutation in the CF pig model. Although the broader vision is to achieve successful base editing across mutations not amenable to small molecule treatments, these studies will allow a direct comparison to modulator therapy in a large animal model. These studies are innovative because they incorporate a small gene editor, termed SaKKH Cas9, fused to an adenine base editor which fits within the size constraints of AAV. This approach efficiently restores anion transport in CF pig CFTR-G551D airway cells. Additionally, these studies include formulating AAV vectors in hypertonic saline to boost gene delivery. This formulation method allows delivery of AAV at lower doses to study dose dependent increases in gene editing compared to CFTR-mediated anion transport. Completion of these studies will be an important milestone to inform translatability of base editors for additional CFTR targets.

Cystic Fibrosis-Related Diabetes: Advancing Care for People with CF in an Evolving Landscape

Saturday, July 25, 2:35 pm

Kevin Scully, MB, BCh, BAO, *Brown University / Providence, RI*

Cystic fibrosis-related diabetes (CFRD) remains one of the most consequential comorbidities affecting people with cystic fibrosis (PwCF), and is associated with worsening pulmonary function, nutritional decline, and increased morbidity. While highly effective CFTR modulators have transformed survival and overall disease management for many PwCF, they have also reshaped the clinical landscape of dysglycemia in CF—catalyzing new questions about screening practices, timing of insulin initiation and emerging novel therapeutic strategies. At the same time, our understanding of CFRD pathophysiology continues to evolve, with an increased focus on beta cell dysfunction compounding progressive islet destruction. This presentation will explore the changing paradigm of CFRD screening, diagnosis, and management, highlighting novel approaches to early detection, beta cell preservation, individualized nutrition, and therapeutic intervention in the era of highly effective CFTR modulators.

Kevin Scully, MB, BCh, BAO, is a pediatric endocrinologist at Rhode Island Hospital/Hasbro Children's Hospital and an Assistant Professor at the Warren Alpert Medical School of Brown University. He serves as Associate Director of the Pediatric Cystic Fibrosis Program and leads the CF endocrinology programs within both the pediatric and adult multidisciplinary CF clinics.

Dr. Scully's clinical and research interests center on cystic fibrosis–related endocrinopathies, particularly cystic fibrosis–related diabetes (CFRD). His work has examined the effects of CFTR modulators on dysglycemia, the relationship between continuous glucose monitor–derived metrics and clinical outcomes, the feasibility of hybrid closed-loop insulin delivery systems for CFRD, and the role of low–glycemic load dietary interventions in improving glucose tolerance and body composition. He has been awarded research support from the Cystic Fibrosis Foundation, including the Harry Shwachman Clinical Investigator Award and recognition through the EnVision III: Emerging Leaders in CF Endocrinology program.

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

Saturday, July 25, 2:35 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 is working to identify, characterize, and develop Bcc-targeting phages, including both lytic phages and prophages isolated from the genomes of clinical Bcc isolates. We received requests for phage therapy evaluation from over 45 patients (nearly all pwCF), and evaluated the susceptibility of 120 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, and we are currently working to convert BCC02 to a lytic phage through a variety of approaches. Finally, we are exploring various aspects of phage delivery, including phage+antibiotic interaction testing and phage nebulization. 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, 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 joined the Division of Infectious Diseases at the University of Pittsburgh School of Medicine in 2018. The Van Tyne lab studies how bacteria evolve during human infection and develops phage therapy as a new approach for treating antibiotic-resistant bacterial infections. Dr. Van Tyne has published over 115 research articles and has trained over 50 young researchers, from undergraduate students to postdoctoral fellows. She is the Co-Director of the Center for Evolutionary Biology and Medicine at the University of Pittsburgh School of Medicine, and is the Co-Chair of the Pittsburgh Phage Project.

CF in the Context of the Exposome: Recent Developments and Future Directions

Saturday, July 25, 3:35 pm

Gabriela Oates, PhD, *University of Alabama / Birmingham, AL*

The CF exposome represents the cumulative environmental, social, and behavioral exposures throughout one's life that interact with genetics to shape CF outcomes and explain variability in disease outcomes and progression. Air pollution, tobacco smoke, climate extremes, neighborhood factors, and housing conditions contribute to inflammation, pathogen susceptibility, and accelerated lung function decline. Socioeconomic status, race and ethnicity, family structure, healthcare access, insurance coverage, and newborn screening policies create disparities in CF outcomes, treatment uptake, and survival. Internal biological responses including chronic inflammation, oxidative stress, epigenetic modifications, immune dysregulation, and microbiome disruptions mediate the effects of environmental exposures and underlie disease heterogeneity. Prenatal, early childhood, and adolescent exposures have outsized impacts, and cumulative exposures across the life course synergistically influence long-term health. An exposome approach enables identification of modifiable risk factors, informs precision care, and guides interventions targeting both environmental and social determinants to address disparities in CF outcomes.

Gabriela Oates, PhD, is a social epidemiologist with research expertise in the role of social and environmental factors for CF clinical and popula-

tion health outcomes. She also leads interventions to minimize adverse exposures and support CF self-management. Her work is funded by grants from the National Institutes of Health, the Cystic Fibrosis Foundation, the Robert Wood Johnson Foundation, and the Alabama Department of Public Health.

Dr. Oates is an Associate Professor in the University of Alabama at Birmingham (UAB) Division of Pulmonary, Allergy and Critical Care Medicine and a scientist in the UAB Cystic Fibrosis Research Center, the UAB Center for Outcomes and Effectiveness Research and Education, and the UAB Center for Clinical and Translational Science. She serves as the Director for Population Health Sciences at the UAB Lung Health Center, Director of the UAB Center for the Study of Community Health, and Director of the UAB Social Determinants of Health Institutional Research Core. She has a 21-year-old son with CF diagnosed through newborn screening.

* Development of a Phage-Based Gene Delivery Platform for Restoring Wild-Type CFTR Expression on the Human Lung Epithelia

Saturday, July 25, 3:35 pm

Saumel Perez Rodriguez, PhD,* *Stanford University / Palo Alto, CA*
Paul Bollyky, MD, PhD,* *Stanford University / Palo Alto, CA*

Gene therapy has incredible, yet largely unrealized, potential to improve human health. However, the major obstacle limiting its broader development is efficient gene delivery. Adeno-associated virus (AAV) vectors, the current gold standard for gene delivery, present significant limitations, including strong immune responses and a limited DNA packaging capacity. We recently discovered that fluorophore-labeled Pf4 bacteriophage from *Pseudomonas aeruginosa* is readily internalized by human lung epithelial cells and localizes to intracellular compartments, including the cytosol and nucleus, suggesting its potential as a therapeutic delivery vector.

To investigate this possibility, we isolated through in vitro evolution a Pf4 variant that behaves as a replisome, remains genetically stable, and contains a 5 kb deletion. A suitable genomic insertion site that permits DNA cloning without disrupting phage production was identified in this phage, and used to clone a sfGFP cassette. Recombinant phages were produced and purified by chromatography, and tested in human lung cell lines. However, while control plasmids and AAV vectors produced robust GFP expression, sfGFP-Pf4 phages failed to generate detectable transgene expression. These results indicate that post-entry intracellular barriers limit Pf4-mediated gene delivery.

Nevertheless, the ability of Pf4 phage to access intracellular compartments such as the cytosol presents opportunities beyond gene delivery. We are currently repurposing this Pf4 miniphage platform for the delivery of inhibitory peptides targeting the NF-κB signaling pathway, a key driver of inflammation in airway epithelial cells of cystic fibrosis patients. Such an approach could help mitigate the chronic inflammatory responses that contribute to progressive lung tissue damage.

Saumel Perez Rodriguez, PhD, is a Postdoctoral Research Scholar at Stanford University School of Medicine, where he develops bacteriophage-based platforms for gene and protein delivery to human cells. His current work focuses on engineering phage systems for CFTR restoration in lung epithelial cells as a potential therapeutic strategy for cystic fibrosis.

Dr. Perez Rodriguez earned his BS and MS in Biology and Biotechnology from the University of Havana and his PhD in Biomedical Sciences from the National Autonomous University of Mexico. His doctoral research investigated cellular bottlenecks affecting recombinant protein secretion in CHO cells, integrating cell physiology, bioprocess engineering, and proteomics.

Throughout his training, he has developed broad expertise in molecular biology, immunology, recombinant protein production, viral and plasmid vector optimization, monoclonal antibodies, and murine models. He has also led industry-oriented R&D projects focused on biotherapeutic development and holds four granted patents related to recombinant protein expression technologies.

In addition to his academic work, Dr. Perez Rodriguez has experience in phage engineering, microbial genetic modification, and the development of phage-based products targeting multidrug-resistant bacteria. He is the recipient of multiple fellowships and scientific awards and actively contributes to the scientific community as a peer reviewer. His long-term goal is to establish an independent research program developing phage-based therapeutic and delivery platforms.

Dr. Paul Bollyky (pronounced "boy-key") is originally from Stanford, Connecticut. He majored in biology as Columbia as an undergraduate. He earned his D.Phil at the University of Oxford, and his MD at Harvard Medical School. He completed his residency training at Brigham and Women's Hospital and his Fellowship Training in Infectious Diseases at the University of Washington. Paul joined the Stanford University Medical School faculty in 2013. He is currently the Stanford Medicine Endowed Professor of Infectious Disease and serves as Chief of the Division of Infectious Diseases and Geographic Medicine.

The Bollyky Lab studies bacteriophages, viruses that infect bacteria, with the goal of developing innovative strategies to treat lung infections in CF and other settings. Key areas of active research involve: 1. Bacteriophages and bacterial pathogenesis, 2. Phage therapy to treat antibiotic-resistant bacterial infections, 3. Synthetic biology, specifically the development of phages as vectors for delivery of genes, peptides, and small molecules to human cells.

Sunday, July 26, 2026

Panel: Impacts of Aging with CF

Sunday, July 26, 9:05 am

Ahmet Uluer, DO, MPH – Moderator,
Boston Children's Hospital/ Brigham and Women's Hospital / Boston, MA
Art Brace, PhD, *Belmont, CA*
Julie Desch, MD, *San Rafael, CA*
Laura Mentch, EdM, *Bozeman, MT*

In the United States, over 60% of people with cystic fibrosis are adults. Of these adults, 30% are aged 40 or older. This increase in longevity is largely due to highly effective modulators, improved care, and an increasing number of adults diagnosed later in life. While celebrating this progress, adults with CF face increased comorbidities as they age, including CF-related diabetes, osteoporosis, gastrointestinal and liver disease, cancer, and cardiovascular disease – many of which overlap with general aging issues. During this panel presentation, moderated by a CF pulmonologist deeply committed to improving multidisciplinary care models for adults with CF, a panel of adults with CF will share their personal experiences with aging, including the age-related comorbidities and mental health challenges that accompany this process. Topics explored include chronic pain, cancer, and impacts upon the family. Through personal stories and guided conversation this session will illuminate the evolving care needs of adults aging with CF, including strategies to improve multidisciplinary care.

Moderator and Panelists

Ahmet Uluer, DO, MPH, 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 chronic illness. His MPH degree from Harvard TH Chan School of Public Health expanded his interests and 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 the 'It's a Lung Story' podcast, where they explore the realities of aging with CF in the era of modulators and medical advancements.

Art Brace, PhD, is a retired research scientist living with cystic fibrosis. He received a BA in Biology from Case Western Reserve University and a PhD in Genetics from Stanford University. He spent his scientific career as a researcher at several biotech companies focused on target discovery, early drug discovery, and drug development. When he retired from full-time work, he began volunteering as both a scientific advisor and CF patient advocate for the Cystic Fibrosis Foundation and CFRI. He is a member of the Research Advisory Committee for CFRI and recently joined the Board of Directors. He is on several committees for the CFF including being a patient representative on the Data Safety Monitoring Board for several CF drug development clinical trials and the Adult Advisory Council. Art is passionate about leveraging both his scientific background and lived experience as a CF patient to benefit the CF community. At home he enjoys gardening, cooking, and travel in the US and Europe with his partner Carmen.

Laura Mentch, EdM, was diagnosed with cystic fibrosis at the age of 50, after years of living with diagnoses of asthma and allergies, taking allergy shots for decades. She is the youngest of 5 and the only one with CF. Her career as a health educator helped her prepare for the "job" of living with CF; she gratefully brings her experience and skills from that work to the CF community. Currently she co-facilitates the CFRI support group for adults with a late CF diagnosis with Dr. Jeanie Hanley, coordinates a retreat for mothers of children and adults with CF for the Cody Dieruf Foundation, and is the historian for the United States Adult Cystic Fibrosis Foundation. Originally from upstate New York, she currently lives in Bozeman, MT. She and her partner are parents of 3 adults and grandparents to 2 teenagers.

Julie Desch, MD, serves as the President of the Board of Directors of CFRI and is a member of CFRI's Research Advisory Committee. At 65 years of age, Julie wakes up every morning amazed and grateful to be alive and healthy, breathing with her native lungs despite two copies of F508del. Her interest in CF research led her to Stanford Medical School, where she worked with Dr. Jeffrey Wine in the Cystic Fibrosis Research Laboratory as she pursued her medical degree. She then completed a residency and two fellowships in Anatomic Pathology at Stanford. After training, she worked at Kaiser Hospital in San Francisco, California as a surgical and skin pathologist. After retiring to take better care of herself and to be a full-time mom, she became a certified personal trainer and wellness coach for children and adults with CF before pursuing teacher training in Mindfulness-Based Stress Reduction, followed by a two-year Mindfulness Teacher Training Certification program, and then coach training in Unified Mindfulness. She has taught meditation online for the last ten years. She currently offers four workshops per year for the CF community, focusing on different aspects of mindfulness and how it can help cope with the challenges of CF.

* Towards Overcoming Evolved Bacteriophage Resistance via Next-Generation Directed Evolution

Sunday, July 26, 9:05 am

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

Bacteriophages (phages) offer a promising alternative to antibiotics in people with cystic fibrosis and chronic airway infections. However, therapeutic application is limited by the narrow spectrum of infectable hosts for any given phage and the rapid emergence of phage-resistant mutants (PRMs). Here

we present our next generation directed evolution strategy, where we leverage resistance itself to engineer more effective phages. For this approach, a collection of PRMs is generated through repeated phage exposure to bacterial isolates. The PRMs are then used to train the phages, resulting in an evolved phage collection able to overcome resistance.

PRMs emerge rapidly and are associated with reproducible mutations. Working with clinical isolates of *Stenotrophomonas maltophilia, Staphylococcus aureus*, and *Pseudomonas aeruginosa*, we show that PRMs emerge rapidly and are associated with reproducible mutations in surface and cell wall pathways. Many PRMs showed increased susceptibility to antibiotics, including trimethoprim-sulfamethoxazole and vancomycin.

Next generation directed evolution produces phages that infect both ancestral strains and PRMs. Our early results show that the evolved phage communities exhibit more durable suppression of both ancestral and resistant strains compared to the ancestral phages. In some cases, lytic activity of the evolved phages increased by more than ten million-fold. Ongoing work focuses on genetic and phenotypic characterization of PRMs and evolved phages, and quantifying the durability of resistance suppression.

These findings demonstrate that directed phage evolution against pre-evolved resistant hosts can yield robust therapeutic phages and positions us for broadly applicable phage therapies for anti-microbial-resistant infections.

Sage Dunham, PhD, is a CFRI Elizabeth Nash Postdoctoral Fellow in the laboratory of Prof. Katrine Whiteson, from UC Irvine. Sage's research focuses on developing bacteriophage-based therapeutic strategies for treating antibiotic resistant infections in people with cystic fibrosis. Sage earned his PhD in Chemistry from the University of Illinois at Urbana-Champaign and has worked as an industrial R&D Chemist.

In his free time, Sage enjoys surfing, hiking, skiing, ceramics, working in his garden, and hanging out with his family.

Katrine Whiteson, PhD, 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/**

The Bloom Effect: How Resilience Transforms Us

Sunday, July 26, 11:00 am

Kimberly Morse, MSW, LCSW, *Children's Hospital Los Angeles / Los Angeles, CA*

Living with cystic fibrosis (CF) – or caring for someone who does – means navigating a landscape of psychosocial challenges that extend far beyond depression and anxiety. The emotional, developmental, and social load of CF is broader, more complex, and more interconnected than some people realize. Medical traumatic stress, chronic pain, substance use, and disordered eating may emerge across the lifespan, alongside higher rates of ADHD and increased emotional impacts for those with autism spectrum disorder (ASD). Social and environmental stressors — including racism, discrimination, bullying, identity-related stress, and safety concerns — also shape daily wellbeing and should be a part of any holistic assessment. Resilience is not a luxury only meant for the mentally tough, the strong, or the fearless. Every one of us has the capacity for resiliency, and this can be taught as a practical, learnable skill set. This session explores accessible coping strategies and the role of resilience in navigating

complex experiences. Grounded in five interconnected pillars — self-awareness, mindfulness, self-care, positive relationships, and purpose — participants will gain tools, resources, and insights informed by lived experiences of resilience, to support both people with CF and caregivers.

Kimberly Morse, MSW, LCSW, from Children's Hospital Los Angeles (CHLA), earned her Master of Social Work degree at University of Washington in 2001, with a concentration in Health/Mental Health. Kimberly has spent her social work career working with patients of all ages, with a variety of medical conditions, in medical settings across the U.S. She joined CHLA in 2011.

Kimberly has worked in cystic fibrosis (CF) over the past fifteen years at CHLA and is inspired by the resilience of many patients and families. Kimberly has received CF Foundation grant funding to support mental health and contributed to a publication related to the positive impact of narrative medicine. Kimberly has given multiple presentations within the professional CF community and CHLA. Kimberly is a member of CFRI's Diversity and Inclusion Advisory Committee and has been involved in workshops and presentations that reflect values of DEI. Kimberly is on the NACFC Program Planning Committee after both chairing and speaking at several NACFC sessions. Kimberly has been a CFF Social Worker Mentor for seven apprentices and is a current clinical supervisor and former field instructor for MSW interns, to both support and learn from the next generation of social workers.

* Factors that Promote Antibiotic-Tolerant *Staphylococcus aureus* CF Infections

Sunday, July 26, 11:00 am

Daniel Wolter, PhD, *University of Washington / Seattle, WA*

Staphylococcus aureus is the pathogen cultured most frequently from CF respiratory samples and is associated with worse CF lung disease. *S. aureus* establishes chronic airway infections despite antibiotic treatment, but how *S. aureus* persists is poorly defined. *S. aureus* exhibits remarkable metabolic flexibility and variability, which could help to explain its persistence. The CF airway is also complex and heterogeneous, comprising variable nutrients, oxygen, and other selective pressures that affect bacterial growth, virulence, and antibiotic activity. Therefore, survival of S. aureus during antibiotic challenge likely involves a combination of bacterial and environmental characteristics that influence states of tolerance, dormancy, or resistance. We hypothesize that alterations in bacterial metabolism in response to variations in nutrient availability and stress responses to antibiotics promote *S. aureus* survival during antibiotic challenge.

To test this, we defined ATP levels (a marker for tolerance), proportions of dormant cells (known as persisters), and bacterial genetic content for a panel of laboratory and CF *S. aureus* isolates, comparing those characteristics among isolates that were killed at different rates by the commonly used antistaphylococcal antibiotic trimethoprim-sulfamethoxazole (SXT). We identified genetic and physiological markers that correlated with amounts of SXT-mediated killing, including ATP levels and prophage content. We also found that *S. aureus* survived SXT-mediated killing much better in a medium that mimics CF sputum compared with standard laboratory media, and that this difference was not caused by drug inactivation. These early results demonstrate that bacterial genetics, physiological responses, and airway chemistry play a role in *S. aureus* survival of SXT.

Daniel Wolter, PhD, is a microbiologist and research faculty member at the University of Washington Department of Pediatrics and Seattle Children's Hospital Division of Pulmonary and Sleep Medicine. He also serves as director of the UW Microbiological Outcomes Advancement Core's (UW MOAC) Isolate and Testing Sub-Core, a national resource center that develops and provides special and clinically relevant microbiological tests and resources for CF research and therapeutics development. Dr. Wolter is particularly interested in understanding how bacteria persist and adapt during chronic infections, the selective pressures that promote bacterial adaptations, and the consequences of these changes on clinical outcomes and response to antibiotic treatment. His research has focused on the detection and analyses of pathogenic organisms,

such as *Pseudomonas aeruginosa* and *Staphylococcus aureus,* that cause chronic infections, especially in CF lung disease. With many years devoted to CF research, Dr. Wolter has been awarded and participated in various CF Foundation and NIH supported studies with a singular purpose of improving the lives of people with CF.

The Stories We Inherit: CF, Community, and the Search for Awe

Sunday, July 26, 11:55 am

Tré LaRosa, *Arlington, VA*

At 32 years old, Tré LaRosa is living a life he could only dream of as a kid: Married to the love of his life, who deeply understands CF; working as a scientific project manager and patient engagement leader; and starting to plan for a future as a potential father. As a scientist and writer, Tré realizes that his good health is due to a mixture of good luck, scientific collaboration and brilliance, and a touch of hard work and resolve. But Tré has also seen the worst-case scenario: In 2018, he watched as his sister, Alyssa, only 29 years old, lost her life after a few month bout with chronic rejection after her second double lung transplant.

In his closing speech, Tré will invite the audience to join him on a winding narrative of his life and loved ones, his experience as a CF researcher and patient advocate, his love of reading, the particularly unique aspects of the CF community, the loss of his sister, the future of CF research, and ultimately, why awe and wonder are what life is all about.

Tré LaRosa is a scientific project manager, science writer, and patient advocate based in the Washington, DC area. He currently serves as project manager at the Foundation for the National Institutes of Health, where he co-leads patient engagement efforts and manages the MASHtrack biomarker study, focusing on translational science, biomarker development, and the research infrastructure that moves science from bench to bedside. He holds a degree in chemistry with an emphasis in biochemistry from the University of Kentucky and brings nearly a decade of experience across basic, translational, and clinical research settings.

A person living with cystic fibrosis, Tré has been deeply involved in the CF community throughout his career. He has served on the Cystic Fibrosis Foundation's Clinical Research Executive Committee since 2022 and previously served on the CFF's Adult Advisory Council. He is a regular columnist for CF News Today, where he covers CF science and life with CF.

Tré's work is grounded in a commitment to patient-centric research based on the conviction that integrating the perspectives of people with lived experience is necessary to achieve successful outcomes for the people affected by chronic and rare diseases. He is a former member of the National Association of Science Writers and maintains an active presence in patient advocacy and science communication beyond his work at FNIH.

* Designing a CF Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cells

Sunday, July 26, 11:55 am

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

Gene therapies that restore cystic fibrosis transmembrane conductance regulator (CFTR) gene function would offer life changing treatments for cystic fibrosis (CF) patients. Realizing these therapies is limited by an inability to target and deliver gene editing or CFTR expression inducing reagents to stem cells in the human airway. Here we present the development of broadly applicable nanoscale tools required to restore function of CFTR towards a clinically translatable mRNA therapy. Lipid nanoparticles (LNPs) were designed as delivery vehicles for CRISPR/Cas9 gene editing reagents to enable integration of a codon optimized linear double stranded DNA (ldsDNA) CFTR donor cassette, demonstrating complete restoration of CFTR protein production and function in 16HBEgeG542x model airway cells with just 3.5% CFTR integration. Further optimization of CFTR integration efficiency and application to differentiated airway basal stem cell

(ABSC) models was limited by the ldsDNA loading capacity of LNPs; a challenge we have begun to overcome through incorporation of a multivalent cationic lipid into our LNP formulations, tuning internal LNP structures to better accommodate large, rigid ldsDNA templates, and enhancing mRNA cargo expression twofold. To increase LNP cell specificity, we developed both click-chemistry mediated antibody conjugation and peptide decoration of LNPs to target ABSC surface markers determined by single cell RNA sequencing. These LNPs are being tested in in vitro models utilizing airway submucosal gland organoid models. Development of these nanotechnologies provides the nucleic acid constructs, delivery vehicles, and in vitro models necessary to advance the development of clinically translatable mRNA therapies for CF patients.

Ruby Sims, PhD, is an interdisciplinary scientist whose work has advanced the development of next-generation mRNA-based therapeutics through integration of nanotechnologies for delivery of nucleic acid payloads. She is currently a Postdoctoral Scholar in the Department of Pediatrics at the David Geffen School of Medicine at UCLA, where her research focuses on improving the efficacy, specificity, and accessibility of these precision medicines. Her work with CFRI as a recipient of the Elizabeth Nash Memorial Fellowship centers around developing non-viral lipid nanoparticle (LNP) delivery vehicles to enable safe and efficient delivery of mRNA encoding for the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein. These efforts work towards inducing functional expression of the CFTR protein in airway cells by improving LNP mRNA payload delivery, and LNP-antibody conjugation for targeted delivery to airway stem cell populations.

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. 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. Dr. Jonas' research explores the intersection of pediatrics, hematology/oncology, stem cell biology, bioengineering, and nanoscience & 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 by establishing rapid, safe, cost-effective, and efficient approaches for transporting genetic engineering and genome-editing machinery for manufacturing gene and cell-based therapies directed at pediatric cancers, hematologic disorders, cystic fibrosis, and other childhood diseases. These capabilities advance the laboratory's efforts to create tools that enable scientists to probe and to interact with stem cells more precisely and empower clinicians 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, and the Hyundai Hope on Wheels and Tower Cancer Research Foundations.

2026 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.

Terry Machen, PhD

Dr. Terry Machen is Professor Emeritus of Cell Biology, Development and Physiology in the Department of Molecular and Cell Biology at UC Berkeley. In the Machen lab, he and his team investigated molecular and cellular mechanisms involved in ion and fluid transport across epithelial tissues, especially those in the GI and respiratory tracts. His most recent work focused on the interactions of airway epithelia and bacteria, with particular emphasis on the role of the cystic fibrosis transmembrane conductance regulator in preventing infections. Dr. Machen has authored/co-authored nearly 200 published articles that have appeared in recognized journals such as Frontiers in Cellular and Infection Microbiology, American Journal of Physiology-Lung Cellular and Molecular Physiology, Cell Reports, and Journal of Cystic Fibrosis. Between 1991 and 2009, Dr. Machen received several CFRI research grants to support his innovative research. Funded projects included: chloride transport and membrane traffic in epithelia; CFTR in intracellular organelles and exocytosis; CFTR's gating effects / F508Del Mutation; proton channels and acid secretions in airways; and redox regulation in CF airway epithelia. Dr. Machen advanced our understanding of the disease. As his fellow researcher – and co-author of numerous articles – Beate Illek, PhD, shares, *"Terry's discoveries have had a lasting impact on the field of CF. His research has influenced generations of scientists, shaped concepts of CF disease mechanisms, and laid critical physiological foundations for the development of transformative therapies."*

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.

Missy Peterson

Missy Peterson has been participating in CFRI's programs for many years, but her formal involvement began in 2020 when she joined CFRI's CF Adult Advisory Committee. Diagnosed with CF when she was 1 year old, Missy received a double lung transplant 29 years ago at the age of 23. She is an ardent advocate for organ donation. She is an active participant in the Transplant Games, where she has medaled in multiple sports. In addition to CFRI's CF Advisory Committee, Missy serves on the CF Adult Retreat Committee, where she brings new ideas and good humor to every meeting. Since the launch of our virtual retreats, Missy has played a significant role in serving as a welcoming host for the virtual attendees. Missy is a strong advocate for raising CF awareness and championing legislation and policies that advance access to care. Since she joined the CF Adult Advisory Committee, Missy has engaged with CFRI advocacy activities – first in Colorado and now in South Dakota. She has participated in several CFRI podcasts on her CF journey, transplant, and colon cancer awareness, and she has written several blogs for CFRI's website. Even as she waits for a needed kidney transplant, Missy is always very generous with her time and very supportive of her fellow committee and CF community members. She fully embodies the spirit of this award.

Continued on page 31

Continued from page 30

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.

Steven Freedman, MD, PhD

Steve Freedman is a highly respected clinician and researcher who gives selflessly of himself to improve the lives of those with cystic fibrosis. Dr. Freedman is the 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). 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. Dr. Freedman 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. Based on his successful leadership of the DIGEST Program, Dr. Freedman helped design CFRI's Patrick Nash Fellows Training Program, which is training cohorts of fellows from multiple disciplines to create a new and improved model of adult CF care. He is a member of the Steering Committee, serves as program faculty, and provides mentorship to several post-doctoral fellows. In his lab, Dr. Freedman is exploring the mechanisms that lead to higher pancreatic cancer rates among those with CF. Dr. Freedman is a caring clinician who, despite his busy schedule, makes himself available to his patients and their families.

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.

Haimlata (Hema) Patel

Hema Patel is an extraordinarily dedicated volunteer for CFRI who shares her time in a wide variety of capacities. She became part of the CF community after her son Niraj's late diagnosis as a teenager. Hema serves on CFRI's Diversity and Inclusion Advisory Committee, where her insights regarding outreach to the South Asian community have been invaluable. She seeks to help others avoid her family's experience: her son suffered from CF-related symptoms for many years and was likely not diagnosed earlier because he was of South Asian descent. Her awareness efforts include assisting with translation of materials to Hindi, and the creation of impactful outreach materials. She has served on educational panels and participated in several CFRI-created diversity-related podcasts and films. Hema also serves as a very active member of CFRI's Gala Committee, where her expertise has helped to advance CFRI's fundraising capacity. She has participated as an advocate at CFRI's CF Awareness Day in Sacramento for the past two years, where she shared her story, raised awareness of CF, and advanced legislation with assemblymembers, senators, and members of their staff. Hema is a joy and a delight to work with. She is extremely generous with her time, most especially when it comes to raising awareness and providing tangible resources and support for our community.

Special Thank You

CFRI Professional and Volunteer of the Year Awards Panel

Zoe Davies, NP
Marina Gonzales
Raksha Jain, MD, MSc
Alicia Maciel, MBA
Christine Nash, 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
Siri Vaeth, MSW

CFRI Conference Photographer

Thomas Horal

Creators of our Virtual CFRI Conference Platform

Jeff and Eden Lord

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 and Discussion Groups/Guidelines

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

— *Adults with CF / Including Those Post-Transplant – Sonya Haggett, LCSW*
— *Parents/ Caregivers / Spouses / Partners / Relatives of Someone with CF – Deb Menet, 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 and 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**

Ways to Support CFRI in Pursuing Its 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 17, 2026, in-person at the historic Hillsborough Racquet Club (Hillsborough, CA). Wine, dine, bid in our auction, and dance the night away. Sponsorships are available!

42nd ANNUAL GOLF TOURNAMENT BENEFITTING CFRI — Held on Monday, August 17th, 2026 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 — During the month of May – Cystic Fibrosis Awareness Month – visit CFRI's social channels to explore daily CF facts and Purple Power posts. And for our followers - weekly CF sticker & swag giveaways you won't want to miss. Like, comment & share our posts. Tag @CFRI.cureCF, use #CFRIPurplePower and support CF by engaging, donating and spreading awareness. Every action makes an impact!

DANCE LIKE A FOOL VIRTUAL DANCE MARATHON — Join us in February 2027 for our sixth annual event! Held in memory of CFRI staff member Danielle Mandella, dozens of dancers from across the country log in and dance over a period of 3 hours. Dancers seek pledges and have fun while dancing and supporting CFRI's wellness programs – all from the comfort of 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 Marina Gonzales at mgonzales@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 7:30 pm. Online Support Group for Adults with CF, which is open to participants nationwide and facilitated by a social worker well versed in issues facing adults with CF.

For 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.

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: CF Teen Connection — 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.

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 8 – Mindfulness to Cope with Sadness and Depression

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.

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!

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 2026 Virtual Summer Retreat will be held Friday, August 7 through Sunday August 9. There is no cost to participate: ***Join us!***

CF Wellness Initiative

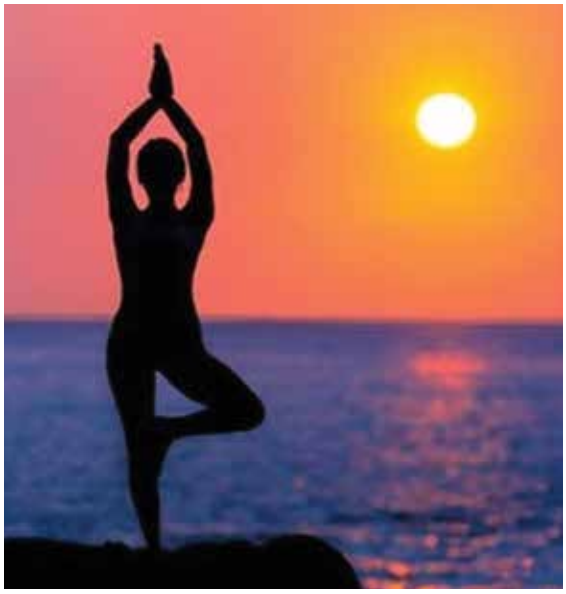
The CF Wellness Initiative helps CF community members to achieve optimal physical and mental wellbeing. Components include Pilates, aerobics, Yoga, QiGong, and CF Strength and Conditioning. Free online classes are held the first and third Saturday of the month and are open to those with CF and their family members. All abilities are welcome.

Purple Power CF Awareness Challenge

Each May during CF Awareness Month, we challenge the community to join us in all things purple – the CF awareness color. We encourage people to visit CFRI's social channels all May long to explore daily CF facts & Purple Power posts. Our followers have opportunities to receive CF stickers & swag giveaways. Participants post their photos on social media, tag CFRI and challenge their friends to join them.

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.



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, colon cancer risks, and more.

Patrick Nash Fellows training Program: Aging In The New Era of CF

The next frontier to extend and improve the lives of people with CF is to address the CF-related comorbidities that present or progress in adulthood, including GI complications, diabetes, bone disease, heart disease, cancer, and others. Named after Patrick Nash, an adult with CF who passed away at the age of 50 due to pancreatic cancer, this educational program for post-doctoral fellows educates and connects the next generation of CF care providers and thought leaders. Through a symposium, mentorship and quarterly meetings, the program serves as a catalyst for innovative multidisciplinary research activities. The 3rd Annual Symposium takes place November 12 – 14, 2026, in Dallas TX.

A Breath of Fresh Air Gala Event

On Sunday, October 17, 2026 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 2026 CFRI Champion, Jacqueline Zirbes, DNP, RN, of Stanford.

CFRI Research Awards

CFRI's investment in new ideas has enabled researchers at academic and medical institutions across the United States to bring new perspectives to the study of this disease. CFRI seeks to fund projects that are original and/or pioneer a new approach to a therapy or cure for cystic fibrosis, through two research programs: the New Horizons Research Campaign (for PIs) and the Elizabeth Nash Memorial Fellowship (for Post-Doctoral Fellows). Researchers are encouraged to submit proposals during the fall funding cycle, to advance the current understanding of cystic fibrosis.

At this weekend's conference, attendees have the opportunity to hear from 10 CFRI-funded researchers, who are pursuing projects related to phage therapy, stem cell research, gene-editing strategies, strategies to address antibiotic resistant pathogens, and more. Much of this research will benefit all those living with CF, regardless of their CFTR mutation.

The above programs are made possible through grants, sponsorships, bequests, foundations, and private donations. Key sponsors include: ***Vertex Pharmaceuticals, Viatris, Sionna Therapeutics, AbbVie, Genentech,****and the* ***Boomer Esiason Foundation****.*

For information about any of these programs, 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.