34th National Cystic Fibrosis Education Conference

July 30 – August 1, 2021

Illuminating the CF Journey
A Fully Virtual Experience
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Greetings

Anna G. Eshoo
Eighteenth District
California

Washington, D.C. 20515

July 30, 2021

Dear Friends,

It is a great pleasure to welcome you to California’s 18th Congressional District, where the Cystic Fibrosis Research Institute is located, and to CFRI’s 34th National Cystic Fibrosis Education Conference, titled “Navigating the CF Journey,” offered in a fully virtual format in light of Covid-19.

Cystic fibrosis is the most common life-threatening genetic disease in children and young adults, affecting their respiratory, digestive and reproductive systems, and it impacts individuals of every race and ethnicity. Since 1975, CFRI has funded innovative cystic fibrosis research, raised public awareness of the disease, and provided vital education and support services to the cystic fibrosis community.

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

On behalf of the people of California’s 18th Congressional District, I thank CFRI for its superb work, and I offer my best wishes for the success of this year’s conference. Always know you have me as your partner in Congress to advance research to find a cure to Cystic Fibrosis.

Most gratefully,

Anna G. Eshoo
Member of Congress
Dear Friends,

Welcome to CFRI’s 34th National Cystic Fibrosis Education Conference, Illuminating the CF Journey. We hope this message finds you well and safe during these ongoing challenging times. We are sorry that once again we are not able to meet in person due to the pandemic, but grateful that this virtual platform has allowed hundreds of people from across the country and globe to participate.

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

Our 2021 conference provides you the opportunity to hear from over 20 experts in the field of cystic fibrosis, addressing mRNA therapies, gene editing, COVID-19, phage therapy, CF diversity, exacerbations, GI issues, mental health, and much more.

Please take the time to explore our virtual conference center. Click on the auditorium doors to choose your live presentations. Please visit our exhibitors – not only can you learn about their products and services, you will earn points for fun drawings. Visit the lounge for Yoga, and the opportunity to connect with others. Don’t forget to take your commemorative photo in the photo booth. And should you need technical or navigational help, our info desk will be staffed all weekend.

Our annual conference also provides us with the opportunity to celebrate heroes in the field. We hope you will join us at our Saturday awards celebration, followed by a virtual dance party.

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

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

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

Warm regards,

Bill Hult
President, CFRI Board of Directors

Siri Vaeth, MSW
Executive Director, CFRI
Attendee Guide
34th National CF Education Conference

Attending CF Education Virtual Conference

Thank you for attending the 34th National CF Education Conference which can be accessed from the comfort of your own home.

Like an in-person conference the CF Education virtual conference will offer a variety of sessions such as: CF Therapeutic Advances, CFRI Funded Research, Awards Celebration & Dance Party, Discussion & Support Groups.

This guide will help you navigate the virtual event to make sure you don’t miss any of the great features!

How to Attend

The virtual conference can be accessed from most up to date computers and mobile devices such as laptops, desktops, and handheld tablets.

On the morning of the event, you will receive a reminder email which contains a link to the login to the CF Education Virtual Conference using your registration information. Once you enter your login information, you will enter our virtual lobby.

You can also access the event by going directly to https://cfri.vizzi.live/ and entering your login information.

If you are having any issues logging in please click on the “Technical Support” button located on the event page.

You can also email Help@virtualcreativestudio.com

Navigating the Virtual Space: Virtual Lobby

The virtual lobby is the hub of the event and will allow you to easily navigate the venue and access the conferences features and sessions.

Networking & Live Chat Tools

Our Networking Lounge is separated into discussion topics. Each topic has its own text chat and video chat where you can network with other attendees, speakers and presenters interested in the same topic.
Here you can view, access & watch all of the sessions. You can also add them to your calendar.

**Exhibit Hall**

Browse exhibitor booths that match your interest, and connect/engage directly with the exhibitors.

**Swag Bag**

Your virtual swag bag will be in the navigation bar on the virtual platform. You can download and save documents for future reference.

**Auditorium**

In the auditorium, you will see “Click Here to View Sessions” located at the bottom of the center screen. This will direct you to the agenda and allow you to choose the sessions that you want to attend. Just before the scheduled start time, you will see a button to join the session.

You can attend as many or as few sessions as you would like, and you can easily navigate between ongoing sessions. After the session has concluded it will be available On Demand, from the same screen.

**Information & Help Desks**

Receive general information on the virtual conference, exhibitors, presenters, sessions, and chat tools.

Questions? Need technical help? Submit a ticket to get support during the conference along with a troubleshooting guide.

**Gamification**

Earn Gamification points while attending presentations, visiting exhibitors and participating in Lounge activities. Follow your progress on our Leaderboard.

**“How To” Guides**

The Exhibit Hall & Networking Lounge feature a “How To” guide in pdf format. Reference the guides to learn more about how to navigate the space and take advantage of all the available features.
Virtual Conference Schedule

All times listed in Pacific Time. Specific presentation times may vary slightly.
Presentations by CFRI-funded researchers are listed in blue.

Friday, July 30, 2021

4:00 pm – 5:00 pm  Mix & Mingle Event
5:00 pm – 5:10 pm  Welcome and Opening Remarks — Siri Vaeth, MSW, CFRI Executive Director
5:10 pm – 6:00 pm  Breath from Salt — Bijal Trivedi, MSc, MA
6:00 pm – 6:10 pm  Break
6:10 pm – 7:15 pm  Breath from Salt Panel — Moderated by Jeanie Hanley, MD
                    (Isabel Stenzel Byrnes, LCSW, MPH; Paul Quinton, PhD; Jeffrey Wine, PhD)

Saturday, July 31, 2021

8:45 am – 9:00 am  Kick-Off — Siri Vaeth, MSW / Introduction of Emcee Jim Hampton
9:00 am – 9:05 am  Welcome — Francis Collins, MD, PhD, Director, National Institutes of Health
9:05 am – 9:35 am  Getting to the After Times – CF and COVID in Year 2 — Richard Moss, MD
9:40 am – 10:25 am Advances in Phage Therapy as a Treatment for Cystic Fibrosis
                    — Benjamin Chan, PhD; Jonathan Koff, MD
10:25 am – 10:35 am 10-Minute Break
10:35 am – 11:20 am mRNA Therapy as a Treatment for CF — Deepika Polineni, MD, MPH
11:20 am – 11:35 am 15-Minute Stretch
11:35 am – 12:20 pm Pulmonary Exacerbations in the Era of Highly Effective CFTR Modulators
                    — D. B. Sanders, MD, MS
12:20 pm – 1:15 pm  Break (Optional Breakout Yoga / Exhibitor Hall / Lounge)
1:15 pm – 2:00 pm  Advancing the GI Frontier for People with CF
                    — Steven Freedman, MD, PhD
                    OR
                    Regional Regulation of CFTR and Ionocyte Expression in Airways
                    — Kenichi Okuda, MD, PhD
2:05 pm – 2:50 pm  Three Perspectives, One Purpose: Why Medicine Needs Memoir
                    — Diane Shader Smith; Maryanne O’Hara; David Weill, MD
2:55 pm – 3:25 pm  Strength-Building with CF — Taylor Lewis, MA, CSCS
3:25 pm – 3:40 pm  15-Minute Break
3:40 pm – 4:30 pm  Until It’s Done for Everyone: Diversity, Inclusion and Equity in CF Care
                    and Research — Jennifer Taylor-Cousar, MD, MSCS
4:30 pm – 5:15 pm  Exhibitor Hall / Lounge Activities / Break
5:15 pm – 6:30 pm  CFRI Awards Celebration with Special Guests
6:30 pm – 8:30 pm  Dance Party
Virtual Conference Schedule

Sunday, August 1, 2021

9:00 am – 9:15 am  Welcome

9:15 am – 10:00 am  **Hearing is Believing: Hearing Health in Persons with Cystic Fibrosis**  
— Angela Garinis, PhD, CCC-A; Ahmet Uluer, DO, MPH  
**OR**  
**A Multi-'Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis**  
— Keehoon Lee, PhD

10:00 am – 10:45 am  **Advances in Gene Therapy and Animal Models for Cystic Fibrosis**  
— John Engelhardt, PhD

10:45 am – 11:00 am  15-Minute Break

11:00 am - 11:45 am  **Reproductive Health in Men and Women with CF: What Do We Know and What Do We Need to Know?**  
— Raksha Jain, MD, MSc  
**OR**  
**Role of Disrupted Airway-Surface Liquid (ASL) pH Regulation in Small Airways in CF Lung Disease Pathogenesis**  
— Xiaopeng Li, PhD

11:45 am – 12:30 pm  **CF and Body Image**  
— Panel moderated by Meg Dvorak, LCSW; Georgia Brown, MLA; Danielle Mandella; Dominic Quagliozzi, MFA

12:30 pm – 12:45 pm  15-Minute Stretch

12:45 pm – 1:30 pm  **My Life with Cystic Fibrosis, Our Unlocked Futures, and Breaking Down Barriers for the Continued Success of the CF Community**  
— Gunnar Esiason, MBA

1:30 pm – 1:40 pm  Event Gamification Prize Winners Announced

1:40 pm – 1:45 pm  Closing Remarks – Siri Vaeth, MSW, CFRI Executive Director

2:00 pm – 3:30 pm  Discussion / Support Groups (See page 36 for listing)

Activity Lounge

*Visit with friends, discover resources, join in Yoga and stretch sessions, and participate in Support and Discussion Groups. Come and say hello.*

**Networking:** Find your friends and make new ones using our chat feature.

**Saturday Wellness Activities – Led by Stacie Reveles:**
— 11:20 am – 11:35 am PT: 15-Minute Guided Stretch  
— 12:20 pm – 12:50 pm PT: 30-Minute Yoga Session  
— 3:25 pm – 3:40 pm PT: 15-Minute Guided Stretch

**Sunday Wellness Activities – Led by Stacie Reveles:**
— 10:45 am – 10:55 am PT: 10-Minute Guided Stretch  
— 12:30 am – 12:45 am PT: 15-Minute Guided Stretch

**Support/Discussion Groups – Sunday, 2:00 pm – 3:30 pm PT**  
See page 36 for listing of groups.

*Wellness Activities Generously Hosted by Vertex Pharmaceuticals*
Sponsors and Exhibitors

CFRI Recognizes Our Generous Sponsors and Exhibitors
For Their Support of the 34th National CF Education Conference

Premiere Sponsor — Vertex Pharmaceuticals

Sustaining Sponsor — Genentech

Diamond Sponsors — Gilead Sciences; Chiesi USA

Platinum Sponsors — AbbVie; Ionis Pharmaceuticals

Silver Exhibitors — PARI; Translate Bio

Bronze Exhibitor — Alcresta Therapeutics; Foundation Care, An AcariaHealth Solution; Matins BioPharma; Maxor National Pharmacy Services; Viatris Pharmaceuticals; Walgreens | AllianceRx Walgreens + Prime

Supporter — Prodigy Press, Inc.

Wellness Activity Sponsor — Vertex Pharmaceuticals

Saturday Night Dance Party Sponsor — Rock CF Foundation

List current as of 07-07-2021. Updates to list available in digital program.
2021 CFRI Award Recipients

The 2021 CFRI CF Professional of the Year Award
— Yelizaveta Sher, MD, FACLP

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

The 2021 David Stuckert Memorial Volunteer of the Year Award
— Marina Gonzales

Marina is the sister of an adult with CF. She has been an active volunteer with CFRI for over a decade in a variety of capacities. She serves on CFRI’s CF Summer Retreat Committee, where she plays a key role in program planning while designing Retreat marketing graphics. In addition to the Retreat, Marina actively participates in CFRI advocacy events, and serves on CFRI’s Gala Committee. Marina is an inspiring and dedicated volunteer; she is a team player who brings warmth, good humor and humility to her work. While Marina initially volunteered with CFRI out of love and support for her brother with CF, she is now a fully integrated member of the community who is beloved by all who know her and have the honor of working with her.

The 2021 CFRI Partners in Living Award in Memory of Anabel Stenzel
— Jacob Fraker, MSW

Jacob is a Legislative Aide for California State Senator Susan Eggman, where he serves as the Legislative Consultant for the CA Legislative LGBTQ Caucus. An adult with cystic fibrosis, Jacob initially volunteered with CFRI, then served as CFRI’s legislative analyst after which he conducted his Master’s internship with CFRI. In 2020 Jacob was hired as the legislative aide for then Assemblymember Eggman, and then joined her staff upon her election to the Senate, where he advocates for the CF, rare disease LGBTQ communities. He is the staff lead for legislation to create a Rare Disease Advisory Council in California. Jacob has furthered CF and rare disease awareness at the state level while consistently supporting CFRI. Jacob embodies the spirit of this award and Anabel Stenzel’s memory.

The 2021 Paul M. Quinton Cystic Fibrosis Research Legacy Award
— Jonathan Widdicombe, PhD

Dr. Widdicombe is Professor Emeritus, Department of Physiology and Membrane Biology, at the University of California at Davis. Altered function of human airway epithelium is at the heart of a number of respiratory diseases, including cystic fibrosis, and the focus of Dr. Widdicombe’s lab is the study of these epithelia. Over twenty years ago, Dr. Widdicombe and his team determined that airway epithelial cultures obtained from patients dying of cystic fibrosis were unable to secrete chloride ions. The health of those with cystic fibrosis primarily declines due to the gradual blockage of airways with sticky mucous secretions, and much of his research has been directed at determining how a defect in chloride ion transport results in this pathology. For many years, Dr. Widdicombe was actively involved with the CFRI’s Research Advisory Committee. Most notably, he established the Elizabeth Nash Memorial Fellowship Program and was the program’s director for many years. Dr. Widdicombe’s research has had – and continues to have – an enduring impact upon the field of cystic fibrosis.
Conference Gamification Prizes

For those seeking a bit of fun while attending the conference, we welcome you to earn Gamification points while attending presentations, visiting exhibitors and participating in Lounge activities.

Gamification Actions and Points:

<table>
<thead>
<tr>
<th>Basic Actions</th>
<th>Point Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Watch General Session or Researcher Presentation</td>
<td>15 per session</td>
</tr>
<tr>
<td>Visit Exhibitor Booths</td>
<td>5 per screen per booth</td>
</tr>
<tr>
<td>View Exhibitor Document (if available)</td>
<td>10 per document</td>
</tr>
<tr>
<td>Watch Exhibitor Video (if available)</td>
<td>10 per video</td>
</tr>
<tr>
<td>Take Exhibitor Survey (if available)</td>
<td>10 per survey</td>
</tr>
<tr>
<td>Chat with Exhibitor (if available)</td>
<td>15 per chat</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Advanced Points Table</th>
<th>Point Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ask a Question During Presentation Q &amp; A –</td>
<td>5 per session</td>
</tr>
<tr>
<td>Complete Speaker Survey</td>
<td>10 per survey</td>
</tr>
<tr>
<td>Watch Welcome Video in Lobby</td>
<td>10</td>
</tr>
<tr>
<td>Participate in Yoga and/or Wellness Activity in Lounge</td>
<td>15</td>
</tr>
<tr>
<td>View CFRI Advocacy Video in Lounge</td>
<td>15</td>
</tr>
<tr>
<td>Attend Awards Celebration</td>
<td>15</td>
</tr>
<tr>
<td>Attend Virtual Dance Party</td>
<td>10</td>
</tr>
<tr>
<td>Complete Conference Outcomes Survey</td>
<td>15</td>
</tr>
</tbody>
</table>

Prize Packs:

If participants are tied, there will be a random drawing of top tied scorers for the following three prizes.

- **First Place** $200 Amazon or Etsy Gift Card (Your Choice)
- **Second Place** $150 Amazon or Etsy Gift Card (Your Choice)
- **Third Place** $100 Amazon or Etsy Gift Card (Your Choice)

Many thanks to our CFRI community members who donated the prizes.
Special Thank You

CFRI expresses its sincere gratitude to the following individuals for their role in bringing this event to fruition.

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

CFRI Professional and Volunteer of the Year Awards Panel
Francine Bion          Danielle Mandella
Barbara Curry          John Mark, MD
Colleen Dunn, MS, RT, CCRD  Robin Modlin, MA
Meg Dvorak, LCSW        Carole Nakamura, MSN, RN, PCCN, CMSRN
Oscar Flamenco, CPA     Ahmet Uluer, DO

Awards Celebration Special Guests

Boomer Esiason
Boomer Esiason enjoyed a 14-year NFL career as a quarterback for the Cincinnati Bengals, New York Jets and Arizona Cardinals. In 1994, he launched the Boomer Esiason Foundation (BEF) after his son, Gunnar, was diagnosed with CF. Since its creation, the BEF has played a key role in supporting CF research and providing programs directly benefiting the CF community.

Singspire Choir with Ashley Ballou-Bonnema
Professional vocalist, adult with cystic fibrosis, founder and executive director of the nonprofit Breathe Bravely, Ashley launched, sINgSPIRE, an innovative approach to battling CF through the art of singing.

Dance Party Host

Emily Schaller — Rock CF Foundation
In 2004 Emily raised CF awareness by throwing a rock show for her friends; in 2007, Rock CF was born. Since then, the Rock CF Foundation has evolved into a multi-faceted organization empowering individuals with CF to live healthy lifestyles.
Profiles

Special Welcoming Remarks

Francis Collins, MD, PHD — Director, National Institutes of Health

Francis S. Collins, MD, PhD, was appointed the 16th Director of the National Institutes of Health (NIH) by President Barack Obama, confirmed by the Senate and sworn in on August 17, 2009. Dr. Collins is the only Presidentially appointed NIH Director to serve more than one administration. In this role, Dr. Collins oversees the work of the largest supporter of biomedical research in the world, spanning the spectrum from basic to clinical research. Dr. Collins is a physician-geneticist noted for his landmark discoveries of disease genes, including the CFTR gene, and his leadership of the international Human Genome Project, which culminated in April 2003 with the completion of a finished sequence of the human DNA instruction book. He served as director of the National Human Genome Research Institute at NIH from 1993-2008.

CFRI Board President — Bill Hult

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

CFRI Executive Director — Siri Vaeth, MSW

Siri Vaeth has been CFRI’s Executive Director since 2018, but her involvement with CFRI began soon after her daughter Tess’ diagnosis with CF in 1995. As a volunteer, she raised funds, chaired the Newsletter Committee, and served on the Board of Directors for 10 years. She joined CFRI’s staff in 2013. Siri brings many years of nonprofit experience to CFRI, including previous positions as executive director of Big Brothers Big Sisters of Santa Cruz County, nonprofit grant writer, and Head Start social worker. In addition to Tess, Siri has a 22-year-old son. She lives in Santa Cruz, California.

Master of Ceremonies — Jim Hampton

Jim Hampton is the father to twin 28-year-old daughters with cystic fibrosis and a 24-year-old daughter who doesn’t have CF. He is the operations manager for three radio stations owned by Alpha Media located in the East San Francisco Bay area. Jim has been involved with CFRI for many years, and is a former member of the Board of Directors.
Georgia Brown, MLA  
*Cincinnati, OH*

Georgia Brown earned a Master of Liberal Arts in 2000 from the University of St. Thomas. She is trained in public speaking, public relations, and journalism. Her volunteer work incorporates those skills to help organizations communicate in a mutually beneficial way with their stakeholders. Brown spends much of her time with the Cystic Fibrosis Reproductive & Sexual Health Collaborative (CFReSHC), working directly with stakeholders to expand its membership and presence in the cystic fibrosis community. She serves as the CFReSHC Patient Partner Lead, chair of the CFReSHC communications subcommittee and publishes a monthly newsletter for members and stakeholders. Georgia dedicates her time to CFRI through participation on the CF Retreat Committee and the CF Adult Advisory Committee.

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

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

John F. Engelhardt, PhD  
*University of Iowa / Iowa City, IA*

John F. Engelhardt is Professor and Head of the Department of Anatomy and Cell Biology at the University of Iowa (UI) Roy J. and Lucille A. Carver College of Medicine. He currently holds the Roy J. Carver Chair in Molecular Medicine and is Director of the UI Center for Gene Therapy and a National Resource Center focused on assisting companies and academic researchers in the development and testing of genetic therapies for cystic fibrosis and other lung diseases. He received his undergraduate degree in biochemistry from Iowa State University in 1985, and his doctorate in human genetics from Johns Hopkins University in 1990.

Research in the Engelhardt laboratory focuses on the molecular basis of cystic fibrosis disease pathologies, and on the development of gene therapies for this disorder. Included are four major research areas of study: 1) lung molecular and cellular biology as it relates to the pathogenesis and treatment of cystic fibrosis (CF) lung disease, 2) the development of viral vectors and animal models to test gene therapy approaches for CF, 3) pathogenesis cystic fibrosis related diabetes, and 4) the study of airway stem cell niches, the regulatory mechanisms that control stem cell proliferation and repair in the airway, and the development of cell-based therapies for CF using stem cells. Dr. Engelhardt’s research has been highly funded by both the National

*Continued on page 14*
Speaker Profiles

Continued from page 13

Institutes of Health, the Cystic Fibrosis Foundation, and corporate sponsored research agreements, with a career total of more than 100 million dollars in research funding. He was also a cofounder of Talee Bio (now Spirovant Sciences), a company made possible by support from the Cystic Fibrosis Foundation and Emily’s Entourage, with a focus on bringing two novel gene therapy products to CF patients.

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

Gunnar Esiason is a cystic fibrosis and rare disease patient leader, who is passionate about early-stage drug development, patient empowerment, antimicrobial resistance, and health policy. After completing his BA at Boston College in 2013, he developed a patient engagement platform for a medical nutrition company, built a venture philanthropy practice at the Boomer Esiason Foundation and was the head coach of his high school alma mater’s varsity hockey team. He has consulted on clinical trial development, a real-world evidence population health study, and a cystic fibrosis-specific mental health and wellness screening tool. During the coronavirus pandemic, Gunnar was a leading voice for equitable vaccine access for people with underlying health conditions. In 2019, Gunnar delivered the pre-commencement address at the St. Louis University School of Medicine commencement exercises and recently published the white paper, Antimicrobial Resistance: Learning from the current global health crisis to prevent another one.

He holds a Master of Business Administration from the Tuck School of Business at Dartmouth and is presently working towards a Master of Public Health at the Dartmouth Institute for Health Policy and Clinical Practice. Gunnar sits on the board of directors at two non-profit organizations, the Boomer Esiason Foundation and No Patient Left Behind. His health policy opinions have been featured in the Wall Street Journal, USA Today, The Hill, and STAT News and among other leading news sources. Follow him on Twitter @G17Esiason.

Steven D. Freedman, MD, PhD
Harvard Medical School; Boston Children’s Hospital/Brigham & Women’s Hospital; Beth Israel Deaconess Medical Center / Boston, MA

Steven D. 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, Boston, Massachusetts. He has played a leadership role in clinical/translational research both at Beth Israel Deaconess Medical Center and at Harvard Medical School 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 (GRASP) of the Harvard Clinical and Translational Science Center, which is a unique longitudinal program that provides project management support and grant writing tools to enhance the transition from the NIH K to R01 grant for junior faculty across the Harvard affiliated hospitals. Finally, he helped establish the CF Foundation funded DIGEST program to train pediatric and adult gastroenterologists in the GI aspects of CF. He also plays a leadership role for the CF Foundation to develop and carry out GI related CF research.

Dr. Freedman received his PhD from Yale University School of Medicine in 1981 followed by the MD degree at the University of Connecticut in 1986. He completed his residency and fellowship in Gastroenterology at Beth Israel Hospital and has remained on faculty since 1991. Dr. Freedman’s expertise is in exocrine pancreatic disease with a particular focus on pancreatitis and cystic fibrosis. He is an internationally recognized leader Continued on page 15
in these areas with an extensive research program that encompasses both basic science discovery as well as clinical trials. More recently, he has expanded these research discoveries to the pathogenesis and treatment of Neonatal morbidities. He has also been a leader in identifying the site in the brain where pancreatic visceral pain is represented, has developed molecular signatures of pain using MR Spectroscopy, and has successfully developed pain therapies using Transcranial Magnetic Stimulation.

**Angela Garinis, PhD, CCC-A**  
*Oregon Hearing Research Center (OHRC); Oregon Health & Science University (OHSU) / Portland, OR*

Dr. Angela Garinis is an Assistant Professor at the Oregon Hearing Research Center at Oregon Health & Science University and a Principal Investigator at the National Center for Rehabilitative Auditory Research at the VA Portland Health Care System. Dr. Garinis received her Master’s degree in Audiology in 2003 and her PhD in Speech and Hearing Sciences in 2008 at the University of Arizona in Tucson.

Dr. Garinis’ research program is focused on the ototoxicity monitoring and management of persons receiving ototoxic treatments, such as aminoglycoside antibiotics. Her current NIH-NIDCD funding [R21DC016128-01A1] is focused on investigating the effects of ototoxic aminoglycoside treatments on the cochlear and medial efferent auditory system in patients with CF, and her CF Foundation Clinical Award (#GARINI19A0) is a multi-center project to investigate the genetic susceptibility or resistance to ototoxicity in patients with CF.

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

Raksha Jain, MD, MSc, is an Associate Professor of Pulmonary and Critical Care Medicine and Director of the Adult CF Care Center at the University of Texas Southwestern in Dallas. She is also Co-Director of the site Therapeutics Development Network program. She has been an adult pulmonologist/intensivist and researcher in the field of CF for nearly 15 years. Dr. Jain obtained her bachelor of science degree at the Massachusetts Institute of Technology in molecular biology and received her medical degree at the University of Texas Houston Health Science Center. She completed her residency and chief residency in internal medicine at UT Southwestern and moved to Washington University in St. Louis for her fellowship in pulmonary and critical care medicine.

Dr. Jain oversees multicenter clinical trials related to cystic fibrosis and conducts her own clinical and molecular scientific research. Her main areas of research revolve around women’s health, health disparities particularly as they relate to sex disparities and sexual and reproductive health.
Speaker Profiles

Jon Koff, MD
Yale University, New Haven, CT

Dr. Koff received his undergraduate degree from Hamilton College and his medical degree from Case Western Reserve University. He completed residency training in Internal Medicine at Brown University. He then completed fellowship training in Pulmonary & Critical Care Medicine at the University of California, San Francisco (UCSF). This included research in the Cardiovascular Research Institute in the laboratory of his mentor, Jay A. Nadel, MD, and dedicated fellowship training in adult cystic fibrosis and lung transplantation. Dr. Koff joined the faculty at UCSF where his clinical responsibilities included: Associate Director of the Adult Cystic Fibrosis Program and member of the Lung Transplantation Program. Dr. Koff joined the faculty at Yale University in 2011 to direct the Adult Cystic Fibrosis Program and to continue his research that focuses on pulmonary immunology.

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

Taylor Lewis is a Clinical Exercise Physiologist and Pulmonary Exercise Researcher. He currently spends his time teaching, training, writing, and researching exercise science to improve the quality of life for individuals with CF through individualized training programs. In 2017, he founded Cystic Fibrosis Fitness Institute, a subdivision for what is now known as Pulmonary Performance Institute (PPI). PPI’s mission is to help improve the quality of life in individuals with a pulmonary condition through exercise.

Prior to founding PPI, Taylor served as the Strength and Conditioning coach for Sonoma State University Baseball, while he pursued a master’s degree in Kinesiology. He is currently pursuing a Ph.D. in Health and Human Performance at Concordia University Chicago. He holds certifications as a Certified Strength and Conditioning Coach (CSCS), Certified Massage Therapist (CMT), Postural Restoration Trained (PRI-PRT), and a Performance Enhancement Specialist (NASM-PES). In addition, Taylor has been a featured speaker at numerous conferences, both in the United States and Internationally, and continuously produces instructional videos about strength and conditioning, and respiratory enhancements for individuals with CF.

Danielle Mandella
Cystic Fibrosis Research Institute / Palo Alto, CA

Dani is 34, living with CF and received a lung transplant in 2003. She has battled pancreatic insufficiency since being diagnosed with CF at three months old due to Failure to Thrive. Since then, strength and weight gain have been constant struggles, leading to crucial realizations and lifelong self-awareness around body image.
Amir Moheet, MBBS
University of Minnesota / Minneapolis, MN

Dr. Amir Moheet is an Associate Professor in the Division of Endocrinology, Diabetes and Metabolism at University of Minnesota. Dr. Moheet graduated from Dow Medical College in Karachi, Pakistan. He completed his Internal Medicine residency training and chief resident year at State University of New York at Buffalo, NY, and his endocrinology fellowship from University of Minnesota in 2012.

He is actively engaged in clinical and translational research. His primary research interest is in the area of diabetes, obesity and its complications. His clinical and research interests include cystic fibrosis related diabetes. In 2016, he was selected to participate in the EnVision CF: Emerging Leaders in Cystic Fibrosis Endocrinology Program by the Cystic Fibrosis Foundation. In his research, he is examining the pathogenesis on hypoglycemia in CF and also developing novel strategies for management of CFRD in obese or overweight patients with CF.

Richard Moss, MD
Stanford University / Palo Alto, CA

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

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

Maryanne O’Hara is the author, most recently, of *Little Matches: A Memoir of Grief and Light* (HarperCollins, 2021). Little Matches is inspired by 9LivesNotes.com, a blog that Maryanne kept while her daughter Caitlin was waiting for a lung transplant. Since Caitlin’s passing, she has been certified by the University of Vermont’s Larner College of Medicine as an end-of-life doula, so that she may better speak to the state of end-of-life care in our culture. Little Matches is a People Magazine Book of the Week, and Maryanne and Caitlin’s story has been featured in TIME Magazine, The Boston Globe, Psychology Today, the New York Times, and elsewhere.

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

Dr. Deepika Polineni is an Associate Professor of Medicine in the Division of Pulmonary and Critical Care Medicine at the University of Kansas Health System, and Associate Adult Program Director for the University of Kansas CF Care Center. She received her Bachelor of Arts and Doctor of Medicine degrees from the University of Missouri, Kansas City, and Master of Public Health degree from Boston University. She completed internship and residency in Internal Medicine at the University of Michigan Ann Arbor, fellowship in Pulmonary Medicine at the University of North Carolina at Chapel Hill, and post-doctoral research in pulmonary medicine at Washington University in St. Louis. Dr. Polineni’s research interests include the identification of non-CFTR genetic modifiers of cystic fibrosis (CF) lung disease, as well as developing methods for improving treatment adherence in CF. Her long-standing research focus has been in-vivo transcriptomic and metabolomic studies to identify differential gene expression and metabolite variability associated with CF lung disease severity. These studies are complementary to ongoing genomic studies of the International CF Modifiers Consortium, with the goal of advancing personalized CF therapies.

Dominic Quagliozi, MFA
*Los Angeles, CA*

Dominic Quagliozi is a 38-year-old visual artist living and working in Los Angeles, CA. He makes artwork that is guided by his lived experience with cystic fibrosis and being a recipient of a double lung transplant. Working across multiple mediums, he presents themes of patient experience, vulnerability, physicality of the body and the intersection of chronic illness and sexuality.
Paul Quinton, PhD
UC San Diego School of Medicine / La Jolla, CA

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

D. B. Sanders, MD, MS
Riley Hospital for Children / Indiana University School of Medicine
Indianapolis, IN

D.B. Sanders, MD, MS is the Associate Director of the CF Center at Riley Hospital for Children and an Associate Professor of Pediatrics at the Indiana University School of Medicine in Indianapolis. Dr. Sanders completed medical school and his pediatric residency at Northwestern University Feinberg School of Medicine. He completed his fellowship in pediatric pulmonary at the University of Washington/Seattle Children’s Hospital. He obtained a Master of Science in epidemiology during his fellowship. After completing fellowship, he was an assistant professor at the University of Wisconsin before moving to Riley Hospital for Children/Indiana University in Indianapolis in 2016. His research focuses on epidemiologic and clinical studies of pulmonary exacerbations, and early life events that contribute to CF lung disease.

Diane Shader Smith
Los Angeles, CA

Diane Shader Smith is a publicist, speaker, writer, cystic fibrosis fundraiser and advocate for bioethics and phage therapy. After her daughter, Mallory Smith, died at age twenty-five from a superbug infection secondary to her cystic fibrosis, Diane compiled and edited Mallory’s diary entries and had them published them as *Salt in My Soul: An Unfinished Life*. Diane has traveled the county speaking passionately and perceptively about her daughter’s writing, sharing profound insights about the hardships of living with chronic illness, the patient experience, organ transplant and phage therapy. Mallory hoped that her writing would help others suffering from the maladies that plagued her: chronic illness, anxiety, depression, body image issues and the troubles of coming of age. And so it does, impelled by Diane’s powerful speaking.
Speaker Profiles

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

Dr. Taylor-Cousar is a tenured professor of adult and pediatric pulmonary medicine at National Jewish Health (NJH), where she serves as the Medical Director of Clinical Research Services, President-elect of the medical staff, and is Co-Director of the Adult CF Program and Director of the CF Therapeutics Development Network (TDN) center. She received her undergraduate degree in human biology from Stanford University in 1993, and completed her doctorate in medicine in 1998, combined residency in internal medicine and pediatrics in 2002, and her combined fellowship in adult and pediatric pulmonary medicine in 2006 at Duke University Medical Center. She obtained her Master of Clinical Science from the University of Colorado in 2015.

She has been site primary investigator on more than 45 clinical studies, and global site investigator on 3 clinical trials. Her investigator initiated research focuses on the development and evaluation of novel therapies for the treatment of CF, and on the unique health needs of women with CF. She is also investigating the etiology and treatment of bronchiectasis in non-human primates. She serves on numerous local and national committees including the CF Foundation’s Clinical Research Advisory Board, the CF TDN’s Clinical Research by Post Hill Press. Executive Committee and as Chair of the CF TDN’s Women’s Health Research Working Group. She recently completed service on the American Thoracic Society (ATS) Scientific Advisory Committee, and is the elected Chair for the 2020-2021 ATS Clinical Problems Programming Committee.

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

Bijal P. Trivedi is an award-winning journalist specializing in longform narrative features about biology, medicine and health. On March 1, 2021, she joined National Geographic as their Senior Science Editor. Before that she was Science and Technology Editor for The Conversation. Last year, in the midst of the COVID-19 pandemic, she completed her first book, “Breath from Salt: A Deadly Genetic Disease, a New Era in Science, and the Patients and Families Who Changed Medicine Forever.” Bill Gates reviewed “Breath from Salt” on his blog and recommended it as one of the top five books for 2020. The book is also on the Longlist for the 2021 PEN/E.O. Wilson Literary Science Writing Award.

Trivedi’s writing has been featured in “The Best American Science and Nature Writing 2012,” National Geographic, Scientific American, Wired, Science, Nature, The Economist, Discover and New Scientist. Her work has taken her to the Mexico-Guatemala border where she covered the use of genetically modified mosquitoes for fighting the dengue virus; to Massachusetts General Hospital where she watched trauma surgeons test hypothermia therapy on pigs with life-threatening injuries; to Moscow’s Star City where she blasted off with space tourism entrepreneurs on the Vomit Comet for astronaut training. She also edited the NIH Director’s Blog and, prior to that, helped launch the National Geographic News Service in partnership with the New York Times Syndicate, which she wrote for and edited. Her undergraduate fascination with biochemistry and molecular biology at Oberlin College compelled her to pursue a Master’s degree in Molecular/Cell/Developmental biology at UCLA. Her love of writing drew her to journalism rather than to a lab bench—and to a second Master’s degree in Science Journalism from New York University.
Ahmet Uluer, DO, MPH  
*Boston Children’s Hospital / Brigham & Women’s Hospital / Boston, MA*

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

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

David Weill, MD  
*Weill Consulting Group / New Orleans, LA*

Dr. David Weill is the former Director of the Center for Advanced Lung Disease and Lung and Heart-Lung Transplant Program at Stanford University Medical Center. He is currently the Principal of the Weill Consulting Group, which focuses on improving the delivery of pulmonary, ICU, and transplant care. Dr. Weill has served in a variety of international and national roles, both in the private and public sectors, and has authored numerous medical articles, book chapters, and editorials. He has twice testified before the United States Senate about how various inhaled occupational exposures affect lung health. Dr. Weill’s writing has appeared in the Wall Street Journal, Salon, Newsweek, the Chicago Tribune, STAT, and the Washington Post. He has been interviewed on CNN and by the New York Times, the San Francisco Chronicle, and the Wall Street Journal. His memoir *Exhale: Hope, Healing, and A Life in Transplant* was published in May 2021 by Post Hill Press.

Jeff Wine, PhD  
*Stanford University / Palo Alto, CA*

Dr. Jeff Wine is the Benjamin Scott Crocker professor of Human Biology and Director of the Cystic Fibrosis Research Laboratory at Stanford University. His research in CF was sparked by his daughter’s diagnosis in 1981. Since 1987, Dr. Wine has studied genetic and cellular aspects of CF in humans and animal models. Dr. Wine and his colleagues identified the first heterozygote effect of the CFTR mutation – discovering that CF carriers have beta-adrenergic sweat rates that are halfway between those with CF and CF carriers – and he adapted that assay to accurately measure levels of CFTR function that are restored by CFTR-directed therapies. His studies of the innate defense system of the airways have helped suggest rationale therapies for keeping CF lungs healthy.
Keehoon Lee, PhD
*The Pathogen and Microbiome Institute, Northern Arizona University*
*Flagstaff, AZ*

Dr. Keehoon Lee completed his PhD in 2017 from Yonsei University, College of Medicine in South Korea and is currently a postdoctoral scholar at the Pathogen and Microbiome Institute, Northern Arizona University working with Dr. Emily Cope. Since he started his research in microbiology in 2003, his studies were mostly focused on medical biofilms related to respiratory health. Keehoon has a broad background in microbiology, genetics, and immunology with specific expertise in biofilm research and respiratory microbiome analysis. His PhD studies in the Department of Microbiology and Immunology at the Yonsei University were focused on characterizing genetics and molecular determinants of *Pseudomonas aeruginosa* mono- and dual-species biofilms. Keehoon has identified a possible new target for *P. aeruginosa* virulence attenuation, the ferrichrome receptor A. He also has succeeded to characterize molecular determinants for the thicker matrix production in dual-species, *P. aeruginosa* and *Enterococcus faecalis*, biofilm. These studies have been published. Currently, his postdoctoral researches investigate the effects of microbial communities in upper and lower respiratory tracts with chronic respiratory diseases. Specifically, the focus of his investigation is to determine relationships with the diseases and bacterial-fungal respiratory microbiota interaction. In his work, he proved the species-specific interactions between predominant mycobti member, *Malassezia*, and major pathobionts in CRS (*P. aeruginosa* and *S. aureus*) can affect host immune response in different manners. This study has been published. He is currently investigating the CF microbiome to determine the specific microbial members that contribute to the respiratory health of CF patients using multi-omics approaches, including shallow shotgun metagenomics and comparative metatranscriptomics. He is also involved in a SARS-CoV-2 serology study and the Alzheimer’s disease and gut microbiome relationship research. He has published more than 16 papers in reputed journals and has been serving as an editorial board member of Brain Sciences, MDPI.

Xiaopeng Li, PhD
*Michigan State University / Grand Rapids, MI*

Dr. Xiaopeng Li received a medical degree from Hunan Medical College in China. He was awarded his PhD from Michigan State University where he was trained in lung injury and repair mechanism. He was trained as a postdoctoral fellow at UCSF for two years to study lung stem cells. Moreover, he got additional training at University of Iowa to investigate the pathogenesis of CF lung diseases using a CF pig model. He had been a faculty member in Iowa as a research assistant professor and then an associate professor for 6 years. Dr. Li is currently an Associate Professor in the Department of Pediatrics and Human Development in the College of Human Medicine at Michigan State University. His main research objective is to investigate the roles of the distal small airways in the pathogenesis of CF lung disease. In CF patients, airway infection and inflammation are the main causes of CF morbidity and mortality. It is generally believed that the small airway abnormalities are involved in early CF lung disease pathogenesis. However, we have more knowledge of how the loss of CFTR function leads to host defense defects in large CF airways, the mechanism of how CF small airway is susceptible to infection and inflammation is not clear. We assume that the mechanism of the abnormalities in small CF airways are the same as large airways. This assumption may not correct as differences in epithelial morphology, cell types and gene expression profile, and lack of submucosal glands and continuous cartilages suggest that small airways are not the same as large airways.

*Continued on page 23*
In fact, using the CF pig model, he established regional differences in CFTR expression, airway-surface liquid (ASL) pH, and bacterial killing in pig large vs. small airway epithelia. He is investigating the mechanisms underlying regional differences in regulation of ASL pH which determines host defense function. In addition, he will investigate if selective modulation of ASL pH in the small airways will prevent or delay the development of CF lung disease.

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

Dr. Okuda obtained his MD degree from Yamagata University in Japan, followed by residency training in internal medicine and fellowship in respiratory medicine. Thereafter, he entered the graduate School of Medicine at the University of Tokyo where he learned advanced techniques in molecular biology, as well as tissue culture and mouse model management. In the second year of graduate school, he applied for postdoctoral training in Dr. Richard Boucher’s laboratory at University of North Carolina at Chapel Hill to engage in studies of airway and mucus biology. Under Dr. Boucher’s supervision, he successfully characterized the regional expression patterns of major airway secretory mucins, MUC5AC/MUC5B, and CFTR/ionocytes in normal and CF human airways. Using this study, he earned his PhD degree in medicine from The University of Tokyo in Japan. Within these works, he developed microdissection techniques for small airway tissue isolation and in vitro small airway epithelial cell culture model comparable to large airways, which has been utilized in the CFRI-funded project. Dr. Okuda’s overall research interest focuses on how the mucociliary clearance (MCC) system is regulated to maintain homeostasis in the lung and how it fails in muco-obstructive lung diseases, including CF. Based on these research interests, his long-term career goal is to work, as a professional investigator, toward a full understanding of the MCC system in the lungs and contribute to the improvement of the prognosis in all patients with muco-obstructive lung diseases.

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

Forest Rohwer is a Fellow of the American Academy for Advancement of Science (AAAS), American Academy of Microbiology (AAM) and Canadian Institute for Advanced Research (CIFAR). He led the development of viromics, which involves isolating and sequencing the RNA/DNA from all of the viruses in a sample. From this data, it is possible to determine what types of viruses are present and what functions they are encoding. Dr. Rohwer uses viromics to study ecosystems ranging from the human body to coral reefs and has shown that most genomic diversity on the planet is viral. Dr. Rohwer has published over 200 peer-reviewed articles, has won numerous national and international awards, and is listed as one of the World’s Most Influential Scientific Minds. He has also published two books: Coral Reefs in the Microbial Seas and Life in Our Phage World.
Support / Discussion Group Facilitators

Richard Barth, PhD
Richard Barth has over twenty years of experience as a psychologist with extensive training and experience in treating insomnia, anxiety, depression, alcoholism, and addictions. He has a private practice in Oakland, California where his treatment approach includes Cognitive Behavioral Therapy, Acceptance and Commitment Therapy, and Mindfulness-Based CT.

Julie Desch, MD
At 60 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 the world of cystic fibrosis 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 continued on at Stanford, completing a residency and two fellowships in Anatomic Pathology. After training, she worked for five years 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. She worked as a wellness coach for children and adults with CF before her interest in mindfulness meditation led her to a teacher training in Mindfulness-Based Stress Reduction, followed by a two-year Mindfulness Teacher Training Certification program, and then a mindfulness coach training in Unified Mindfulness. She has taught meditation online for the last four years. Her current online offering, “Mindfulness 2.0,” is a user-friendly course tailored to the CF community. Julie serves on the Board of Directors at CFRI and serves as Chair of CFRI’s Research Advisory Committee.

Meg Dvorak, LCSW
Ms. Dvorak is a licensed clinical social worker who has been with the Adult Cystic Fibrosis team at Stanford since 2010. In addition to working directly with CF patients in clinic, Ms. Dvorak helped develop a peer mentoring program for CF adults, and partnered with the Stanford Adult CF Advisory Board to create and moderate a Stanford sponsored Facebook page for CF Adults. Ms. Dvorak serves on CFRI’s CF Quality of Life Committee. She regularly facilitates support groups with the Embrace Mothers Retreat. Ms. Dvorak has participated in several CFRI podcasts on CF and mental health. She facilitates CFRI’s monthly support groups for caregivers of children and adults with cystic fibrosis, as well as a support group for adults with CF. Ms. Dvorak received CFRI’s Professional of the Year award in 2016 for her service to the CF community.
Support / Discussion Group Facilitators

Jacob Fraker, MSW
Jacob is a Legislative Aide for California State Senator Susan Eggman, where he serves as the Legislative Consultant for the California Legislative LGBTQ Caucus. An adult with cystic fibrosis, Jacob has been involved with CFRI for many years, previously serving as CFRI’s legislative analyst. Prior to his work with Senator Eggman, Jacob served as her legislative aide in the Assembly. In his current role, Jacob advocates for the CF, rare disease and LBTQIA+ communities. Jacob has a BA in Social Work from San Jose State and his Masters in Social Welfare from UC Berkeley.

Sonya Haggett, LCSW
Sonya is a licensed clinical social worker from the San Francisco Bay Area living with cystic fibrosis, who is five years post-double lung transplant. She has served CFRI over the years as group facilitator for the Summer Retreat and Educational Conference and as a Summer Retreat committee member. Her clinical practice has focused on community mental health where medical, criminal justice, and aging issues intersect.

C. Virginia O’Hayer, PhD
Virginia O’Hayer is a Clinical Associate Professor in the Department of Psychiatry and Human Behavior at Thomas Jefferson University Hospital. She formerly served on faculty at Duke University as the Medical Psychology Liaison to the Lung Transplant Team. Dr. O’Hayer is extensively trained in Acceptance and Commitment Therapy, CBT, Dialectical Behavior Therapy, and Radically Open Dialectical Behavior Therapy. She created the ACT with CF manual, and with a grant from the Boomer Esiason Foundation, is conducting a 3-year multi-site randomized controlled trial of ACT with CF vs supportive psychotherapy. Virginia lives in Bryn Mawr, PA with her husband, 2 kids, and 2 rescue mutts: Sandbox and Dogbert.

Alanah Rosenbloom, MSW
Alanah is an adult living with CF. She has a BA in Communication from UC Davis and recently earned a Master’s in Social Work from San José State University with a concentration in gerontology. She is honored to serve her peers as a support group facilitator at this year’s Conference. Alanah has grown alongside CFRI at each stage of her life; from appearing in a fundraising campaign in the 1980’s, to attending the Conference as a teenager, and loving the Adult Retreat into her 20’s and 30’s. Alanah is an only child with several “inherited” nieces and nephews she adores. Outside of CF, she admires animals, reads non-fiction, and likes listening to podcasts.
Support / Discussion Group Facilitators

Yelizaveta Sher, MD
Liza Sher has been a part of Psychosomatic Medicine/Consultation Liaison Psychiatry (CLP) Faculty at Stanford since 2013, where she also serves as an Associate Director of CLP Fellowship and is a Clinical Associate Professor of Psychiatry. Her areas of clinical and research interests include psychiatric comorbidities in patients with pulmonary disorders and she specializes in mental health of patients with CF as well as lung transplantation. Dr. Sher is the first Director of Psychiatric and Psychological Services in the Adult CF Program at Stanford. She has written numerous articles and book chapters, and has co-edited several books. Dr. Sher is CFRI’s 2021 CF Professional of the Year.

Wellness Sessions

Stacie Reveles
Stacie Reveles is a yoga instructor, mother of a child living with CF, and the Advocacy & Programs Associate at CFRI. She approaches this shared practice as an opportunity to start where you are and absorb the balance, rejuvenation, and clarity that yoga can bring.

Panel Moderators

Isabel Yuriko Stenzel Byrnes, LCSW, MPH
Isabel is a bereavement social worker at Mission Hospice, where she counsels and leads writing groups for those who are grieving. She has lived with cystic fibrosis (CF) for 48 years and received a double lung transplant in February, 2004. Isabel has been an active leader for various CF and organ transplant organizations for over two decades. Isabel and her late twin published the memoir, “The Power of Two,” and served as international patient advocates in her mother’s country, Japan, which led to the creation of a documentary film of the same title. Isabel has lectured around the country on topics such as living well with illness, end-of-life issues and organ donation, including a TEDx Stanford talk in 2014.
Panel Moderators

Julie Desch, MD
At 60 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 the world of cystic fibrosis 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 continued on at Stanford, completing a residency and two fellowships in Anatomic Pathology. After training, she worked for five years 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. She worked as a wellness coach for children and adults with CF before her interest in mindfulness meditation led her to a teacher training in Mindfulness-Based Stress Reduction, followed by a two-year Mindfulness Teacher Training Certification program, and then a mindfulness coach training in Unified Mindfulness. She has taught meditation online for the last four years. Her current online offering, “Mindfulness 2.0,” is a user-friendly course tailored to the CF community. Julie serves on the Board of Directors at CFRI and serves as Chair of CFRI’s Research Advisory Committee.

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Jean Hanley, MD
Dr. Jeanie Hanley is a physician with cystic fibrosis and serves on the CFRI Board. She has been married for 35 years and is mother to three adult children. Although she had CF symptoms from the time she was young, she was diagnosed in her early 30’s only after genetic testing became available. Three of her nine siblings also have CF. 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. Advocating for patients and families has been a passion of hers. As a result, Dr. Hanley founded a nonprofit patient advocacy organization called Planning Health. She also is 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 and devotes time to committees involved in Diversity & Inclusion, Research Advisory, and Advocacy.
Presentation Abstracts

Breath from Salt
Bijaal P. Trivedi, MSc, MA
National Geographic, Washington, DC

Friday, July 30, 5:10 pm

In 2011, I knew little about cystic fibrosis, and what’s more, I had never met anyone who suffered from it. It was only once I was assigned to write a magazine feature on Kalydeco, in 2012, that I unwittingly began a journey to tell the complete story of this disease: from its characterization to the discovery of the defect in the cell and gene to the development of the amazing medicines that are available today. And while the science was always path-breaking, it was the individuals in this story who captivated me, won my heart, and made me realize that I really needed to write a book about this epic saga. In this presentation, I’d like to tell you about my decade-long journey writing this book, some of the people who inspired me, and share a few excerpts of Breath from Salt that were particularly moving to report.

Getting to the After Times – CF and COVID in Year
Richard Moss, MD
Stanford University, Palo Alto, CA

Saturday, July 31, 9:05 am

If it feels like it’s been a long year, well, for one thing, that’s because the Year Like No Other has lasted 16 months, since early March 2020 when SARS-CoV-2 landed on the USA like a hammer blow. Not to mention lockdown, masks, six feet apart, isolation, remote school, farewells over Facetime, universal grief, widespread anger, distrust, disinformation, conspiracies, bitter polarization, racial reckoning, a contested election result, an insurrection, democracy on the knife’s edge . . . on and on the list goes.

As the CF community looks forward, it is clear that the USA is emerging from the worst of the Covid-19 threat that has caused 7-13 million excess deaths worldwide and killed 600,000 Americans, but we also face a host of new challenges. In this update I will focus on some key questions about aspects of the pandemic currently relevant to CF: How have people with CF fared? Is having CF really a vulnerability for Covid? Is it clear we really know how you catch SARS-CoV-2? What vaccines are available and are more on the way? Just how good are the vaccines in protecting from serious illness or death? What about protection from asymptomatic Covid infection (which means you could unknowingly give the disease to others)? How safe are the vaccines, and are there important differences between them? How common are breakthrough infections to those vaccinated? What are variants of concern, and how well do the vaccines protect against them? Can vaccinated people spread the virus to unvaccinated others? Do we need to vaccinate children? How risky is reopening of society without masks—what about the unvaccinated? What about transplant recipients and the immunocompromised? Will CF care be changed by our adaptations, for example are telehealth and home monitoring here to stay? Are there medicines that effectively treat Covid? Will booster shots be needed? How common and serious are chronic “long haul” Covid symptoms? I will review current evidence and recommendations as we emerge from this pandemic, and hopefully the lessons learned will help us face the next one, which all experts agree is inevitable in our crowded technologically sophisticated global civilization.

Advances in Phage Therapy as a Treatment for Cystic Fibrosis
Benjamin Chan, PhD; Jonathan Koff, MD
Yale University, New Haven, CT

Saturday, July 31, 9:40 am

Despite the exciting clinical impact of CFTR modulators, clinicians and patients continue to struggle to treat multi-drug resistant (MDR), and increasingly pan-drug resistant (PDR), bacteria. Increased prevalence of MDR and PDR pathogens, which are associated with increased morbidity and mortality, is compounded by a limited antibiotic development pipeline. Thus, development of new antibiotics and management strategies is urgently needed. Because of these issues, there has been renewed interest in bacteriophage (phage) therapy, which has been used to treat infections since their discovery in the early
**Presentation Abstracts**

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Phages are viruses that specifically infect and kill bacteria. Phages are ubiquitous, and found wherever bacteria are present. In addition, there is an opportunity to find naturally occurring lytic phages that kill CF pathogens (e.g., *Pseudomonas aeruginosa* and *Staphylococcus aureus*), or potentially genetically modify these phages to improve their function. In the US there is increasing experience with individual cases and several clinical trials to develop phage therapy for CF. We are excited about a personalized inhaled phage therapy approach that uses phages to target specific bacteria found in individual CF patient sputum. In a cohort of 9 adults with CF, phage nebulized daily for 5 to 10 days decreased *Pseudomonas* sputum bacteria load significantly. In addition, this single phage strategy has been developed to force surviving *Pseudomonas* to be less antibiotic resistant or virulent. This strategy takes advantage of bacteria’s evolutionary ability to develop resistance to phage. For example, phages are selected that target specific virulence factors (e.g., efflux pump or pil) and when exposed to these phages *Pseudomonas* will down-regulate these phage receptors to survive. The result is decreased efflux pump, which leads to decreased MDR or PDR *Pseudomonas*, or to bacteria that are less virulent because they lack pil. This strategy has been deployed in the 9 CF patients who received nebulized phage for *Pseudomonas*. In addition to decreased sputum bacteria load, there was evidence for 1) increased antibiotic sensitivity in individuals that received efflux pump-targeting phage, 2) decreased pilus function, which resulted in less virulence, and 3) an improvement in lung function after phage therapy. These results prompted the development of a clinical trial [Cystic fibrosis bacterioPHage study at Yale (CYPHY)], funded by the CF Foundation and Yale University, to investigate the safety and efficacy of this nebulized personalized phage therapy approach, and the establishment of a Center for Bacteriophage Research and Therapy at Yale.

**mRNA Therapy as a Treatment for CF**

Deepika Polineni, MD, MPH

University of Kansas Medical Center, Kansas City, KS

Cystic fibrosis (CF) is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene yielding defective CFTR protein. These underlying defects result in dysfunctional ion channel transport leading to hyperviscous mucus in the lungs resulting in chronic infection, inflammation and lung function decline. Recently approved CFTR modulator therapies, which correct and/or potentiate defective CFTR, have fundamentally altered the landscape of CF treatments for the majority of people with CF in the United States. However alternative strategies to improve CFTR function are yet needed. It is estimated that for at least 9% of people with CF, modulator treatments are not applicable based on CFTR genotype or adverse effects to therapy. Messenger ribonucleic acid (mRNA) therapy presents a cutting-edge treatment option under investigation for people with CF, independent of CFTR mutation. mRNA is a type of ribonucleic acid that is present in human cells and represents one step in the process of DNA genetic code becoming translated to protein. Using mRNA as therapy in CF involves the careful delivery of mRNA, coding for CFTR, into the cell to use the cells machinery to create normal CFTR protein. Notably, mRNA therapy is not the same as either gene editing or gene therapy. However, much like these other nucleic acid therapies, mRNA has the potential to address all people with CF irrespective of their CFTR genotype. This presentation will provide a review of mRNA as a novel therapeutic option for people with CF of all mutation types, and results of the most recent clinical trials evaluating mRNA therapy in CF will be discussed.
Presentation Abstracts

Pulmonary Exacerbations in the Era of Highly Effective CFTR Modulators  
D.B. Sanders, MD, MS  
Indiana University School of Medicine, Indianapolis, IN

People with cystic fibrosis (CF) have periodic pulmonary exacerbations, which are characterized by an increase in respiratory symptoms above baseline including increased cough, sputum production and decreased exercise tolerance, and decreases in pulmonary function. Pulmonary exacerbations are treated with antibiotics and increased frequency of airway clearance, but are associated with progression of lung disease, poorer quality of life, increased healthcare expenses, and, in people with CF with advanced lung disease, mortality.

The introduction of highly effective CFTR modulators has greatly reduced the frequency of pulmonary exacerbations, although they have not been eliminated entirely. It is also not yet known if CFTR modulators prevent the poor outcomes that can occur after pulmonary exacerbations. One report indicates that ivacaftor does not prevent the loss of lung function that may occur after a pulmonary exacerbation. The long-term impact of highly effective CFTR modulators on pulmonary exacerbations will not be known for years. Furthermore, many people with CF are not able to experience any of these potential benefits if they are not eligible, or can’t tolerate, highly effective CFTR modulators.

In the meantime, research efforts are ongoing to optimize the treatment of pulmonary exacerbations. Investigators have explored treatment options for small degrees of lost lung function in hopes of preventing the development of respiratory symptoms and more severe pulmonary exacerbations. The recently completed STOP-2 trial provided valuable information on the benefits of varying durations of intravenous antibiotics. STOP PEDS is exploring whether children with CF with mild pulmonary exacerbations can avoid antibiotics entirely, especially if they are receiving highly effective CFTR modulators. The impact of the COVID-19 pandemic on CF care has also highlighted the contributions of viral infections to pulmonary exacerbations, as well as options for home monitoring of pulmonary exacerbations including telehealth and home spirometry.

The impact of highly effective CFTR modulators may mean that “a cold is just a cold,” and not something that can lead to weeks of symptoms, missed school or work, or prolonged hospital stays. This talk will provide an overview of these potential benefits and how the CF community is preparing to treat respiratory symptoms in this new era.

Advancing the GI Frontier for People with CF  
Steven Freedman, MD, PhD  
Beth Israel Deaconess Medical Center, Boston, MA

We are making major strides in improving the lung disease in patients living with CF. However, the GI manifestations in these patients is now being recognized as a major cause of symptoms. In fact, multiple studies keep getting the same result – that approximately 80% of our patients living with CF, have GI issues which are frequently moderate to severe and impact the quality of life. The localization of CFTR in epithelial cells lining the GI tract as well as in the nerves regulating motility of the gut, would explain the diverse expression of CF resulting in inspissated secretions, impaired motility, and dysbiosis. This pathophysiology explains the myriad of GI conditions from acid reflux to motility disorders such as gastroparesis as well as constipation and DIOS. Exocrine pancreatic insufficiency is an early manifestation in over 90% of our patients with CF and was thought to be irreversible. However, studies indicate that exocrine pancreatic function can potentially be rescued with highly effective CFTR modulators, at least up to age 5. Similarly, recurrent pancreatitis in pancreatic sufficient patients with CF may be prevented with ivacaftor. As we move down the GI tract, there is an increased risk of colon cancer associated
with CF which can be explained by the lack of normal CFTR function as a tumor suppressor gene, the high fat diet our patients consume, and the altered GI microbiome. Whether Trikafta can reverse the CF manifestations across all organ systems in patients age 12 and older, is being examined through the 2 year long PROMISE trial. As we move into our next frontier to address the GI manifestations of CF, the CF Foundation DIGEST program was created in 2014 to train pediatric and adult GI physicians across North America to expand our multidisciplinary CF care team and create best practice guidelines. Living with CF does not mean living with the GI manifestations.

**Three Perspectives, One Purpose: Why Medicine Needs Memoir**  
*Saturday, July 31, 2:05 pm*  
Diane Shader Smith; Maryanne O’Hara; David Weill, MD  
Los Angeles, CA; Ashland, MA; New Orleans, LA

Patient stories offer valuable insights that go way beyond statistics and outcomes: they have the power to inspire, humanize, compel action, and challenge assumptions. Patients want to tell their stories but how do you choose the right stories, and how do you make sure that the process of telling and hearing them is beneficial to both provider and patient? Diane Shader Smith, Maryanne O’Hara and David Weill, will speak for the patient, the parent, and the provider respectively, and address these questions in the context of cystic fibrosis.


Attendees will learn why narrative medicine is a growing field and why narrative in medicine is so critical. They will leave feeling inspired to share their stories armed with tools for how to better communicate with their CF teams.

**Strength-Building with CF**  
*Saturday, July 31, 2:55 pm*  
Taylor Lewis, MA, CSCS  
Pulmonary Performance Institute, San Rafael, CA

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

CFRD Management Through Technology
Amir Moheet, MBBS
University of Minnesota, Minneapolis, MN

Saturday, July 31, 2:55 pm

Cystic fibrosis-related diabetes (CFRD) is the most common extrapulmonary complication of cystic fibrosis. Around 40% of individuals with CF above age 20% and 50% above age 30 have CFRD. The prevalence is even higher in patients with severe genotypes. The pathophysiology of CFRD is complex but insulin insufficiency or inadequate insulin secretion is thought to be the primary defect. CFRD is associated with increased morbidity and mortality. The current guidelines recommend insulin as the only pharmacological therapy for management of CFRD. The benefits of insulin therapy in patient with CF include improved glucose control, increased weight, improved lung function, decreased frequency of pulmonary exacerbations and improved survival.

To meet the recommended calorie requirement, patients with CF eat multiple times during the day. In patients with CFRD multiple daily bolus injections may be needed to cover these meals and snacks. Patient are also asked to monitor glucose levels which requires frequent finger stick glucose monitoring. This requirement of multiple daily injections and frequent finger stick glucose monitoring ads to the already high treatment burden in these patients.

Recent advancement in diabetes technologies including ambulatory insulin pumps, continuous glucose monitoring (CGM) and hybrid closed-loop insulin delivery systems have transformed the landscape of diabetes management. These technologies are being increasing utilized in the management of CFRD. In this presentation, current insulin pump, CGM and hybrid closed loop insulin options will be discussed. Existing literature on the use of these technologies and their potential benefits in the management of CFRD will be reviewed.

Until It’s Done for Everyone: Diversity, Inclusion and Equity in CF Care and Research
Jennifer Taylor-Cousar, MD, MSCS
National Jewish Health, Denver, CO

Saturday, July 31, 3:40 pm

When Dr. Dorothy Andersen described cystic fibrosis (CF) as a pathological entity in 1938, she noted that of the 39 cases, there were patients of both Puerto Rican (n=1) and African-American (n=1) descent, and that the “racial and demographic distribution of [cases] was wide.” However, until recently, CF has most often been described as a “White” disease. Such characterization has contributed to late diagnosis and treatment in Black, indigenous and people of color (BIPOC) with CF. Late diagnosis and treatment is associated with worse health outcomes including lower lung function and increased risk of death. Furthermore, missed and late diagnoses contribute to medical trauma and compound historical reasons for distrust of the medical system. Both medical system distrust and investigative team implicit bias likely explain the disproportionately low enrollment of BIPOC with CF in clinical trials. Additionally, BIPOC with CF have a lower likelihood of being eligible for cystic fibrosis transmembrane conductance regulator (CFTR) modulators. Based on recent data from the U.S. CF Foundation patient registry, while more than 90% of White people with CF (pwCF) are eligible for a CFTR modulator, only 76% of Hispanic and 70% of Black pwCF are eligible for these life-changing therapies. In vitro evaluation of use of modulators for CFTR mutations more commonly found in BIPOC with CF and not previously included in testing efforts is underway.

Recent acknowledgment of health care disparities in CF by CF organizations and within the CF community is an important first step in addressing the institutional and interpersonal factors that uniquely adversely impact the quantity and quality of life for BIPOC with CF. Efforts to increase representation of diverse voices from the CF community in care and research and CF workforce diversity are important additional steps and are ongoing. Intentionality in our endeavors is crucial to ensure that CF is ultimately cured for everyone.
Presentation Abstracts

Hearing is Believing: Hearing Health in Persons with Cystic Fibrosis  
Sunday, August 1, 9:15 am
Angela Garinis, PhD, CCC-A; Ahmet Uluer, DO, MPH  
Oregon Health & Science University, Portland, OR;  
Boston Children’s Hospital/Brigham & Women’s Hospital, Boston, MA

Persons with cystic fibrosis (CF) are routinely treated with aminoglycoside and glycopeptide antibiotics to manage life-threatening Pseudomonas and other gram-negative bacterial infections. Incorporating audiology care into the cystic fibrosis (CF) care pathway is critical for monitoring potential adverse events to the auditory system (i.e., ototoxicity) from these treatments. Ototoxicity symptoms may include hearing loss, tinnitus (ringing in the ears), vertigo and even speech in noise processing issues. Although monitoring for ototoxicity has not been common practice in CF care centers across the US, emerging evidence has shown high rates of ototoxicity in this patient cohort (up to 57% have hearing loss) and the immediate need for audiological management.

Susceptibility to ototoxicity may vary across patients due to various risk factors such as genetic markers for hearing loss, noise exposure and other concomitant treatments beyond aminoglycosides and glycopeptides administered intravenously. Also, aminoglycoside and glycopeptide dosing based on individualized pharmacokinetic drug monitoring may provide effective antimicrobial activity while minimizing ototoxicity. As treatments for CF improve, patients will be living longer lives with an emphasis on quality of life and thus management of ototoxicity symptoms is critical to address, along with the need for amplification and other aural rehabilitation options.

The present talk will discuss: (i) the pathogenesis of CF with focus on the microbiome and use of ototoxic treatments (e.g., aminoglycosides) (ii) synergistic ototoxic effects (e.g., noise, inflammation, other treatments) (iii) ototoxicity monitoring and management options for CF care centers and (iv) a brief discussion on novel clinical ototherapeutic trials.

Advances in Gene Therapy and Animal Models for Cystic Fibrosis  
Sunday, August 1, 10:00 am
John Engelhardt, PhD  
University of Iowa, Iowa City, IA

Cystic fibrosis (CF) patients have benefited greatly from CFTR modulators, small-molecule drugs that correct the basic defects in the mutant CFTR channel. However, 10% of CF patients harboring CFTR mutations that produce little or no protein will require gene therapy. The two primary approaches for CF gene therapy include gene replacement (the addition of a functional CFTR gene or mRNA) and gene editing (the correction of CFTR mutations within the patient’s DNA or mRNA). Each of these approaches require delivery vehicles (vectors) that move genetic cargo into cells of the target organ (e.g., epithelial cells of the lung in CF patients). Several vector systems are currently being pursued including lipid nanoparticles, RNA viruses (lentivirus), and DNA viruses (adeno-associated virus and human bocavirus). Animal models of disease are integral to the developmental pipeline of gene and cell therapies.

The past decade has seen tremendous advances in CF research that are driving the development of promising genetic therapies. Critical to these advances has been the development of larger CF animal models, which are clarifying the mechanisms underlying lung disease pathophysiology and defining the cellular targets for gene therapy. For two decades, CF mice had been the only animal model available in which to develop therapeutics, but they did not spontaneously develop lung disease because their lung cell biology differs from that of humans. More recently, a Noah’s Ark of new CF animal models was developed, including pigs, ferrets, sheep, rabbits, and rats. Among these, the ferret and pig models have been studied extensively and reproduce the multiorgan CF disease phenotype seen in humans, including the lethal bacterial colonization of the lung, and are being used to test gene therapy approaches. Through the National Ferret Research and Resource Center at the University of Iowa, we have focused
on building a toolbox of CF ferret models that can assist the field in testing new therapeutic strategies for CF lung and pancreatic disease. CF is a complex disease and the path to developing effective gene therapies will likely encounter unknown challenges and setbacks. However, many companies and academic laboratories have contributed significant resources and talent to tackling these challenges and ensure eventual success. As was the case for CFTR modulators, when genetic treatments for CF patients emerge, they will define a path for tackling other complex devastating diseases.

Reproductive Health in Men and Women with CF: What Do We Know and What Do We Need to Know?  
Raksha Jain, MD, MSc  
University of Texas Southwestern, Dallas, TX

Cystic fibrosis (CF) is an autosomal recessive genetic disorder impacting approximately 80,000 people of world-wide. CF is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene which encodes a chloride channel. CFTR protein dysfunction results in abnormal chloride and bicarbonate transport in the respiratory, gastrointestinal and also in the reproductive tracts. As people with CF are leading longer and healthier lives, sexual and reproductive health is becoming an increasingly important topic with a number of unique and unaddressed questions. It is critical that we provide high quality evidenced-based data to men and women with CF on topics including contraception, fertility, pregnancy and lactation, and parenthood. This session will highlight what is known and unknown about male and female infertility in CF, contraception and its impact on health in CF, and pregnancy, lactation and parenthood for men and women with CF. Importantly, this topic will be discussed in the context of highly effective CFTR modulators, which are now available for over 90% of people with CF.

My Life with Cystic Fibrosis, Our Unlocked Futures, and Breaking Down Barriers for the Continued Success of the CF Community  
Gunnar Esiason, MBA  
Boomer Esiason Foundation, New York, NY

Told through the lens of critical conversations with care providers over a lifetime of encounters with the American healthcare system, Gunnar Esiason will describe his journey with cystic fibrosis from childhood to a newly minted M.B.A. Gunnar was diagnosed with cystic fibrosis when he was two years old at Cincinnati Children’s Hospital, and not long after his parents founded the Boomer Esiason Foundation. Initially the organization was set up to raise money alongside the Cystic Fibrosis Foundation’s therapeutic research network, but eventually evolved into philanthropy aimed not only at advancing cystic fibrosis science, but also to provide financial assistance and resources to people with cystic fibrosis and their families at various lifetime milestones, like college scholarships, transplant cost assistance, disaster relief, and, most recently, pandemic economic hardship.

Gunnar grew up in a time with cystic fibrosis when the standard of care had very little variation from patient to patient. New treatments were discovered, but their power waned over time until their efficacy diminished in parallel increasingly stubborn antibiotic resistant infections. By 2018, Gunnar stood on the precipice of end-stage illness after living through a cycle of requiring intervention or hospitalization every other month, if not more frequently, before enrolling in a pivotal clinical trial that saved his life. Gunnar’s eyes have turned to the future of cystic fibrosis, where the next generation of patients will not have to grow up with the same disruptions that Gunnar and patients in his generation have had to endure.
Regional Regulation of CFTR and Ionocyte Expression in Airways  
Kenichi Okuda, MD, PhD  
University of North Carolina, Chapel Hill, NC

**Rationale:** Identification of cells expressing the cystic fibrosis transmembrane conductance regulator (CFTR) is required for precision medicine therapies for cystic fibrosis (CF). However, a full characterization of CFTR expression in normal conducting airways is lacking. We aimed to identify cell types that contribute CFTR expression and function along the proximal-distal axis of normal human lung.

**Methods:** scRNA-seq was performed on human mainstem bronchial epithelial cells obtained from four healthy volunteers by bronchoscopic brush biopsy, and small airway (< 2 mm in diameter) epithelial cells obtained from three non-smoker lung transplant donors by micro-dissection. Complementary single cell-based quantitative PCR (scqRT-PCR) and RNA in situ hybridization (scRNA-ISH) were performed to detect low abundant CFTR transcripts at a single cell level. Intra- and inter-regional expression of CFTR and FOXI1, which is a marker of ionocytes, were examined in normal and CF human lungs by RNA-ISH. In vitro human large (LAE) and small airway epithelial (SAE) cell cultures were utilized for functional assessment of CFTR protein in each airway region. Lentiviruses were used for cell-type specific transduction of wild-type CFTR in CF cells.

**Results:** scRNA-seq identified mucous-secretory cells as dominating CFTR mRNA expression in normal human large and particularly small airways, followed by basal cells. Ionocytes expressed the highest CFTR levels per cell but were rare, while expression in ciliated cells was low. scqRT-PCR and scRNA-ISH confirmed scRNA-seq data. RNA-ISH in normal lungs exhibited discordance between CFTR expression and ionocyte localization in the distal airways. CF lungs demonstrated distribution patterns of CFTR and ionocyte similar to normal controls. CFTR-mediated Cl- secretion in culture tracked secretory cell, not ionocyte, densities. Further, the nucleotide-purinergic regulatory system that controls CFTR-mediated hydration was associated with secretory cells, and not ionocytes. Lentiviral transduction of wild-type CFTR produced CFTR-mediated Cl- secretion in CF airway secretory cells but not ciliated cells.

**Conclusions:** In normal human airways, CFTR expression in mucous-secretory cells suggests the presence of a mechanism whereby airway mucin secretion is coupled to airway surface liquid volume regulation. In the small airway, the combined function of these mucous-secretory, CFTR-expressing cells are likely critical for preventing mucous obstruction and supporting normal MCC.

Personalized Phage-based Therapies  
Forest Rohwer, PhD  
San Diego State University, San Diego, CA

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

A Multi-‘Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis  
Keehoon Lee, PhD  
The Pathogen and Microbiome Institute, Northern Arizona University, Flagstaff, AZ

Pulmonary inflammation due to chronic infections is a leading cause of morbidity and mortality in cystic fibrosis (CF). In the airways, microbes overtly colonize the mucosal surfaces of patients with the disease,
and the composition and diversity of these communities directly relate to airway health status. Previous studies of the CF microbiome using 16S rRNA gene sequencing revealed that a diverse microbiome colonizes the CF patient’s lungs during childhood and its diversity decreases as the disease progresses. CF patients often develop chronic rhinosinusitis (CRS) prior to the pulmonary disease. Recently, several studies have shown that the possible connection between the upper and lower respiratory tract microbiome composition of CF patients with CRS. For example, identical strains of *P. aeruginosa* have been detected in both upper and lower respiratory tracts of CF patients. A recent study of the airway microbiome demonstrated a loss of microbiome niche specificity between the sinuses and lungs of CF patients with CRS, indicating a shared microbiome between the two sites. However, the microbial functional attributes that influence the pathogenesis of the upper and lower respiratory tracts of CF patients are still unclear. In this study, we hypothesize that the specific microbial strains or encoded functions drive concurrent type I inflammation in upper and lower respiratory tracts of CF patients. To test the hypothesis, we used metagenomics and metatranscriptomics on paired upper and lower airway samples from healthy, non-CF-CRS, and CF-CRS patients with various comorbidities. This elucidates the microbial community composition to the strain level, the microbiota functions, and the host immune responses in the upper and lower airways of the subjects with different disease statuses. Our metagenomics results showed a certain strain of *Staphylococcus aureus*, RF122, is associated with polyposis of the patients with CRS, but there was not any particular association with the fungal microbiome and CRS or CF. The beta-diversity analyses also demonstrated significant differences between control, CRS, and CF respiratory microbiome. Furthermore, it presented no significant difference between the upper and lower respiratory microbiome which represents the possible microbial sharing between the two locations. The transcriptome analysis results demonstrated significantly different gene expressions between CF and CRS. The differentially expressed genes are mainly related to inflammatory and immune responses. The findings of this research helped us better understand the relationship between microbiome composition and host response, such as specific gene expressions and their functions in disease control.

**Role of Disrupted Airway-Surface Liquid (ASL) pH Regulation in Small Airways In CF Lung Disease Pathogenesis**

**Xiaopeng Li, PhD**

*Michigan State University, Grand Rapids, MI*

In a newborn pig cystic fibrosis (CF) model, the ability of gland-containing airways to fight infection was affected by at least two major host-defense defects: impaired mucociliary transport and a lower airway-surface liquid (ASL) pH. In the gland-containing airways, ASL pH is balanced by CFTR and ATP12A, which respectively control HCO3- transport and proton secretion. We found that, although porcine small airway tissue expressed little ATP12A, the ASL of epithelial cultures from CF distal small airways (diameter <200 μm) were nevertheless more acidic (compared to non-CF). Therefore, we hypothesized that gland-containing airways vs. small airways control acidification using distinct mechanisms. Our microarray data suggested that small airway epithelia mediate proton secretion via ATP6V0D2, an isoform of the V0d subunit of the H+-translocating plasma membrane V-type ATPase. Immunofluorescence of small airways verified the expression of the V0d2 subunit isoform at the apical surface of Muc5B+ secretory cells, but not ciliated cells. Inhibiting the V-type ATPase with bafilomycin A1 elevated the ASL pH of small airway cultures, in the presence or absence of HCO3-, and decreased ASL viscosity. These data suggest that, unlike large airways, which are acidified by ATP12A activity, small airways are acidified by V-type ATPase, thus identifying V-type ATPase as a novel therapeutic target for small airways diseases.
Support & Discussion Group Guidelines

CFRI’s virtual Support and Discussion Groups offer an opportunity to gather together with CF community peers to share experiences and information that are unique to those touched by cystic fibrosis.

This year we are offering the following groups:

— Mindfulness – Julie Desch, MD
— Body Image – Meg Dvorak, LCSW
— Post-Transplant (limited to adults with CF post-transplant) – Sonya Haggett, LCSW
— Romantic Relationships (limited to adults with CF) – Alanah Rosenbloom, MSW
— Advocacy and CF – Jacob Fraker, MSW
— Medical Regimen Adherence Issues – C. Virginia O’Hayer, PhD
— Newly Diagnosed – Rick Barth, PhD
— Sibling & Family Dynamics – Yelizaveta Sher, MD

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. Our focus is on encouraging and supporting one another. We do not offer individual or group therapy in the support groups, and this is not an opportunity for counseling, diagnosis, or treatment of specific disorders.

• Please be prepared to commit a minimum of 45 minutes with your selected group.

• Confidentiality is important to all attendees. To ensure confidentiality, you are asked to not reveal participants’ names or their personal issues outside of the group.

• There will be a facilitator for each group whose biographical information is listed in the conference program. Facilitators are licensed and practicing professional counselors. They are required by law to report incidences of child, elder or spousal abuse.

• Respect the members of your support group, including their situations, emotions and perspective. Limit making suggestions to others unless they ask for ideas and advice.

• Please give quieter members an opportunity to share.

• It is okay to listen and remain silent. Simply say, “pass,” if people are going around the group 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.
An introduction to some frequently used cystic fibrosis related terms:

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

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

**aerosol** — a mist for inhalation, usually containing medicine.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

genotype — the genetic makeup of a cell (i.e. the specific allele of the individual cell), usually with reference to a specific character under consideration. Delta F508 is the most common CF genotype in Caucasian patients.

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

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

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

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

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

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

IgE or IgG — a type of antibody level found in the blood that indicates exposure to certain allergens or an immune response.
malabsorption — poor uptake of nutrients from food. In CF, mucus may plug ducts of digestive organs and block the secretion of enzymes and hormones, leading to malabsorption.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

pathogen — a microbe or microorganism such as a virus, bacterium or fungus.
percussion — an airway clearance technique that involves clapping on the chest with a cupped hand, or vibrating the chest with another device, to loosen mucus in the lungs.

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

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

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

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

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

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

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

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

sputum — mucus from the lungs; phlegm.

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

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

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

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

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

Partners in Living ~ Research for Life

DONATE TO THE JESSICA FREDRICK MEMORIAL CF RESEARCH CHALLENGE FUND
Thanks to our generous Jessica Fredrick Memorial CF Research Challenge Circle donors, any gift made to the Jessica Fredrick CF Research Challenge Fund will be matched 100%. All contributions will be restricted to our CF research awards in 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.

MOTHER’S DAY CELEBRATION — Our Mothers’ Day Celebration supports our research, education and advocacy programs. Send colorful invitations to friends, colleagues and family members, or participate via our virtual campaign. Please contact us if you would like to become a Sender. It is fast, easy and very meaningful!

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

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

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

— The 37th Annual Golf Tournament Benefitting CFRI at the beautiful top-rated Pasatiempo Golf Club in Santa Cruz on Monday, August 9, 2021.
— CFRI’s Virtual Gala, “A Breath of Fresh Air,” will be held Saturday, October 16, 2021. Sponsorships are available!

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

PURPLE HAIR CHALLENGE — Dye your hair purple during the month of May to raise CF awareness and challenge others to do the same. This fun – and visually pleasing – challenge raises awareness of cystic fibrosis and funds for CFRI’s services.

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 cocktail party, organize a walk-a-thon, or come up with your own creative way to build strength and support for the CF community. Come up with an idea and we will support you!

For more information, please contact Stacie Reveles at sreveles@cfri.org.
CFRI Programs and Events

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

**CF Quality of Life (CFQoL) Financial Support for Individual Therapy**
CFRI underwrites up to $120 per session for six sessions of counseling with the licensed therapist of your choice. This nationwide service is available to children and adults with CF as well as to their family members (siblings, spouses partners, and parents) until annual funds are expended.

**Mindfulness 2.0 Online Class**
Open to participants nationwide and taught by Julie Desch, MD, CFRI’s online class is based on Unified Mindfulness and adapted for the CF community. It shares a new approach in bringing mindfulness into daily life to help those with CF and their family members address anxiety, depression and pain.

**CF Caregivers Online Support Group**
CFRI hosts two online CF Caregivers Support Groups on the third Tuesday of every month. Facilitated by a CF social worker, this group provides peer-to-peer support to help families cope with the daily challenges of life with CF. Parents of children with CF meet at 5:00 pm PT. Parents and partners of adults with CF meet at 6:00 pm PT.

**Adults with CF Online Support Group**
CFRI provides a monthly Online Support Group for Adults with CF, which is open to participants nationwide. The group is facilitated by a social worker well versed in issues facing adults with CF. The group meets on the third Monday of every month, from 6:00 pm PT to 7:30 pm (9:00 pm – 10:30 pm ET).
Navigating Grief to Growth
An online discussion and support group for those who have lost a loved one to CF, whether recently or in the past. Held on the 1st and 3rd Tuesday of each month and led by Isabel Stenzel Byrnes, LCSW, MPH, a grief counselor who also lives with CF, the group provides a safe place to engage in focused practices to help experience growth in coping with the loss of a loved one.

CFRI’s CFQoL Programs are generously supported by Gilead Sciences, Vertex Pharmaceuticals, Genentech, Chiesi USA, Ionis, Viatris, Translate Bio, and private donors.

Many Voices ~ One Voice CF Advocacy and Awareness Program
Our Advocacy and Awareness Program broadens understanding of the physical, emotional, and financial challenges faced by the CF community while seeking to reduce barriers to medical care and therapies and increase investment in research. We need your voice; please get involved!
Generously sponsored by Vertex Pharmaceuticals, Gilead Sciences, AbbVie, Ionis Pharmaceuticals, and Genentech.

CF Summer Retreat
The annual CF Summer Retreat enhances education, positive coping skills, and social support for people who share common experiences with CF, and includes educational presentations, exercise, arts and crafts, support groups, and much more. The 2021 retreat will be held virtually August 19 – 22. Join us!
Generously sponsored by AbbVie, Vertex Pharmaceuticals and Gilead Sciences.

Embrace Retreat for Mothers of Children and Adults with Cystic Fibrosis
The Mothers Retreat provides peer support and expert speakers addressing CF-related resources, self-care for caregivers, stress reduction strategies, and other topics pertinent to coping with chronic illness. Held virtually in 2020 and 2021, it is hoped to be held onsite in Menlo Park, CA in May 2022.
Generously sponsored by AbbVie, Vertex Pharmaceuticals and Gilead Sciences.
CFRI Programs and Events

CF Community Voices Video Podcast Series
Created by and for the CF community, CFRI’s video podcast series is available on our Podbean and YouTube channels. Personal and professional CF experts address diverse topics including nutrition, financial planning, mental health, innovative research, reproductive health, COVID-19, and more.
Generously sponsored by Chiesi USA, Genentech, Viatris, Gilead Sciences and Vertex Pharmaceuticals.

CF Wellness Initiative
The CF Wellness Initiative consists of three complementary multidisciplinary programs to help CF community members to achieve optimal physical and mental wellbeing. Components include Physical Therapy, Yoga and CF Strength and Conditioning. Free online classes are ongoing.
Generously sponsored by Ionis Pharmaceuticals, Viatris, Vertex Pharmaceuticals, and Translate Bio.

Purple Hair Challenge
Each May during CF Awareness Month we challenge the community to dye your hair purple – the CF awareness color – with dye or using a phone app. Participants post their photos on social media with #purplehairchallenge, tag CFRI and challenge friends to join them.
Generously sponsored by Vertex Pharmaceuticals and Chiesi USA.

A Breath of Fresh Air Virtual Gala Event
On Saturday, October 16, 2021, join us for our virtual gala and support the search for a CF cure. In addition to inspiring stories, musical performances, and celebrity appearances, we will honor our 2021 CFRI Champion.
Generously sponsored by AbbVie, Chiesi USA, Genentech, Gilead Sciences, Heritage Bank, Ionis Pharmaceuticals, Translate Bio, Viatris, Vertex Pharmaceuticals, and private donors.

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