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Greetings

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

Anna G. Eshoo
Eighteenth District
California

July 29, 2022

Dear Friends,

It is a great pleasure to welcome you to California’s 18th Congressional District and to CFRI’s 35th National Cystic Fibrosis Education Conference, Focus on the Future.

Cystic fibrosis is the most common life-threatening genetic disease in children and young adults, affecting their respiratory, digestive, and reproductive systems. 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 weekend’s 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 everyone 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 for cystic fibrosis.

Always my best,

Anna G. Eshoo
Member of Congress
Dear Friends,

Welcome to CFRI’s 35th National Cystic Fibrosis Education Conference, Focus on the Future. We hope this message finds you well and safe as we navigate the third year of the pandemic. Once again we regret that we are not able to meet in person this year, but grateful that this virtual platform allows hundreds of people from across the country and globe to participate and engage.

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 2022 conference provides you the opportunity to hear from over 20 experts in the field of cystic fibrosis, addressing mRNA therapies, gene editing, phage therapy, bone health, emerging pathogens, CF and cancer, 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
35th National CF Education Conference

Attending CF Education Virtual Conference

Thank you for attending the 35th 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 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 29, 2022
4:00 pm – 5:00 pm  Mix & Mingle Event
5:00 pm – 5:10 pm  Welcome and Opening Remarks — CFRI Executive Director
5:15 pm – 6:05 pm  Finding the Perfect Predator: The Story Behind the First Dedicated Phage Therapy Center in North America — Steffanie Strathdee, PhD (author of The Perfect Predator)
6:10 pm – 7:10 pm  Panel: Phage Therapy Clinician and Recipients: Saima Aslam, MBBS, MS / Ella Balasa, BS / Thomas Patterson, PhD; Moderated by Julie Desch, MD

Saturday, July 30, 2022
8:45 am – 9:00 am  Welcome — Siri Vaeth, MSW, CFRI Executive Director
9:00 am – 9:05 am  Keynote Remarks — Janet Woodcock, MD, Principal Deputy Commissioner of the FDA
9:10 am – 9:15 am  Introduction — Emcee Jim Hampton
9:15 am – 10:05 am  CF Airway Infections: To Treat or Not To Treat? Why and How? — Dao Nguyen, MD.CM, FRCP(C)
11:05 am – 11:15 am  10-Minute Stretch
11:15 am – 12:05 pm  Advances in Gene Therapy for Cystic Fibrosis — Paul McCray, MD
12:15 pm – 1:05 pm  The Challenges of Aging for People with CF — Richard Simon, MD
1:05 pm – 1:45 pm  mRNA therapy for People with Cystic Fibrosis: Next Steps — Manu Jain, MD, MSCI
1:45 pm – 2:35 pm  Oral Health Considerations for Individuals with CF — Donald Chi, DDS, PhD
2:45 pm – 3:35 pm  Nanotechnologies to Enable Cystic Fibrosis Gene Therapies — Steven Jonas, MD, PhD
3:35 pm – 3:50 pm  15-Minute Stretch
3:50 pm – 4:40 pm  Panel: The Impact of CF on the Siblings — Marina Gonzales / Suraj Patel / Damian Rice; Moderated by Deborah Menet, LCSW
4:40 pm – 5:15 pm  Break / Exhibitor Hall / Lounge Activities
5:15 pm – 6:15 pm  CFRI Awards Celebration with Special Guests
6:30 pm – 8:30 pm  Dance Party
# Virtual Conference Schedule

**Sunday, July 31, 2022**

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>9:00 am – 9:15 am</td>
<td>Welcome — CFRI Executive Director — Introduce Emcee</td>
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<tr>
<td>9:15 am – 10:05 am</td>
<td><strong>Partnership in CF Care: Communication and Collaboration When Considering Complimentary Therapies</strong></td>
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<tr>
<td></td>
<td>— Hanna Phan, PharmD, FCCP, FPPA</td>
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<tr>
<td></td>
<td><strong>Selective Targeting Distal Small Airways by AAV Vectors: Implications for Gene Therapy and CF Lung Disease Pathogenesis</strong></td>
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<tr>
<td></td>
<td>— Xiaopeng Li, PhD</td>
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<tr>
<td>10:15 am – 11:05 am</td>
<td><strong>CF-Related Bone Disease: Current Evidence and Future Directions</strong></td>
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<td></td>
<td>— Melissa Putman, MD, MMSc</td>
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<tr>
<td>11:05 am – 11:20 am</td>
<td>15-Minute Stretch</td>
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<tr>
<td>11:20 am - 12:10 pm</td>
<td><strong>Advocacy in the CF Realm</strong></td>
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<td></td>
<td>— Jacob Fraker, MSW, Diane Shader Smith</td>
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<tr>
<td></td>
<td><strong>Novel Models of CF Mucus Plugs for Testing Phage and Tailocin Therapy</strong></td>
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<tr>
<td></td>
<td>— Gregory Burkeen, PhD Candidate</td>
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<tr>
<td>12:10 pm – 12:45 pm</td>
<td>Break / Exhibitor Hall / Lounge Activities</td>
</tr>
<tr>
<td>12:45 pm – 1:35 pm</td>
<td><strong>Expanding Opportunities for Lung Transplant for Individuals with Cystic Fibrosis</strong></td>
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<td></td>
<td>— Joseph Pilewski, MD</td>
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<tr>
<td></td>
<td><strong>Pseudomonas aeruginosa Diversifies Inside Cells to Form an Adaptive Niche that Resists Antibiotic Treatment</strong></td>
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<td></td>
<td>— Naren Gajenthra Kumar, BTech, PhD</td>
</tr>
<tr>
<td>1:45 pm – 2:35 pm</td>
<td><strong>The Power of Science, Community and Desperation to Speed Lifesaving Breakthroughs for the Final 10%</strong></td>
</tr>
<tr>
<td></td>
<td>— Emily Kramer-Golinkoff, MBE</td>
</tr>
<tr>
<td>2:35 pm – 2:45 pm</td>
<td>Event Gamification Prize Winners Announced</td>
</tr>
<tr>
<td>2:55 pm – 4:30 pm</td>
<td>Support Groups (Adults with CF / Parents / Caregivers of Children with CF / Parents-Spouses-Partners of Adults with CF)</td>
</tr>
</tbody>
</table>
Sponsors and Exhibitors

CFRI Recognizes Our Generous Sponsors and Exhibitors For Their Support of the 35th National CF Education Conference

Premiere Sponsor — Vertex Pharmaceuticals
Sustaining Sponsor — Genentech
Diamond Sponsors — Gilead Sciences; Chiesi USA
Platinum Sponsors — AbbVie; Ionis Pharmaceuticals
Gold Exhibitor — Gilead Sciences
Bronze Exhibitors — Alcresta Therapeutics; Foundation Care, An AcariaHealth Solution; Eloxx Pharmaceuticals; Viatris; Walgreens | AllianceRx Walgreens Prime
Organizational Exibitors — Cystic Fibrosis Engagement Network (CFEN); CF Roundtable; Emily’s Entourage; Rock CF Foundation
Supporter — Prodigy Press, Inc.
Wellness Activity Sponsor — Vertex Pharmaceuticals
Saturday Night Dance Party Sponsor — Rock CF Foundation

List current as of 07-12-2022. Updates to list available in digital program.
The 2022 CFRI CF Professional of the Year Award
— Dennis Nielson, MD, PhD
Dr. Nielson is a Professor of Clinical Pediatrics at UC San Francisco (UCSF), where he served as Director of the Cystic Fibrosis Center at UCSF Benioff Children’s Hospital from 2001 until his recent retirement. Dr. Nielson earned a medical degree as well as a PhD in Physical Chemistry at the University of Utah. He went on to complete a pediatric residency and pulmonary training at UCSF Medical Center, and later organized a new division of pediatric pulmonary medicine at the University of Utah, where he served on the pediatrics faculty for 18 years. After two years in Ohio, he returned to UCSF Benioff Children’s Hospital in 2001. Dr. Nielson has been the principal investigator and participating investigator on numerous clinical trials and his published research has increased understanding of the disease process and potential new therapies. Dr. Nielson serves on CFRI’s Medical Advisory Committee. For his service to CFRI and the CF community, he received CFRI’s 2015 CF Champion Award.

The 2022 David Stuckert Memorial Volunteer of the Year Award
— Anna Payne
Anna Payne’s involvement with CFRI began when she participated in CFRI’s Externally-Led Patient Focused Drug Development Meeting with the FDA. Since then, she has actively served on CFRI’s CF Adult Advisory Committee. Last year, Anna was diagnosed with stage-4 colon cancer. She has been a vocal advocate for CF and colon cancer awareness, and has participated in CFRI podcasts and articles on this topic. Anna serves on the Pennsylvania Rare Disease Advisory Council and as an elected Township Supervisor. Anna has used her own cancer battle to educate others, with hopes they will avoid her fate.

The 2022 CFRI Partners in Living Award in Memory of Anabel Stenzel
— Sonya Haggett, LCSW
Sonya is a licensed clinical social worker from the San Francisco Bay Area living with cystic fibrosis, who is six years post-double lung transplant. Her clinical practice has focused on community mental health where medical, criminal justice, and aging issues intersect. She has served CFRI over the years as group facilitator for the CF Adult Retreat and Educational Conference on the Board of Directors and as a Retreat Committee member. Since 2021, she has facilitated CFRI’s monthly online support for CF community members post-transplant. Sonya embodies the spirit of this award and Anabel Stenzel’s memory.

The 2022 Paul M. Quinton Cystic Fibrosis Research Legacy Award
— Richard Moss, MD
Richard B. Moss, MD, Professor Emeritus of Pediatrics at Stanford University, 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. Dr. Moss received his BA from Columbia, his medical degree from SUNY Downstate, did his residency at Northwestern/Children’s Memorial, and fellowships at Stanford in allergy-immunology and pulmonology. 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. Dr. Moss has published over 250 research papers and is a frequent speaker at national and international medical conferences. He has been a strong supporter of CFRI over the decades and has served on CFRI’s Board of Directors since 2015. For his many contributions to the field of CF and the CF community, Dr. Moss received the CF Champion award in 2017. Dr. Moss’ research has had – and will continue 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</td>
<td>10 per document</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Advanced Points</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>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Advanced Points</th>
<th>Point Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>— Participate in Yoga and/or Wellness Activity in Lounge</td>
<td>10</td>
</tr>
<tr>
<td>— Attend Awards Celebration</td>
<td>10</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 Gift Card
Second Place: $150 Amazon Gift Card
Third Place: $100 Amazon Gift Card

Activity Lounge

A place to visit with friends, discover helpful resources, participate in Yoga and guided stretch sessions, and in Support and Discussion Groups.

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

Saturday Wellness Activities — Led by Stacie Reveles

11:05 am – 11:15 am 10-Minute Guided Stretch
1:05 pm – 1:35 pm 30-Minute Yoga Session
3:35 pm – 3:50 pm 15-Minute Guided Stretch
6:30 pm – 8:30 pm Saturday Post-Awards Celebration Virtual Dance Party

After all the sitting, join your friends from around the nation and globe on the dance floor!
Our virtual dance party is for everyone!

Sunday Wellness Activities — Led by Stacie Reveles

11:05 am – 11:20 am 15-Minute Guided Stretch
12:10 pm – 12:40 pm 30-Minute Yoga Session
3:30 pm – 5:00 pm Sunday Support / Discussion Groups
Adults with CF / Parents, Caregivers of Children with CF / Parents, Spouses, Partners of Adults with CF

Please see pages 23 and 24 for facilitator profiles.

Wellness Activities Generously Hosted by Vertex Pharmaceuticals
CFRI expresses its sincere gratitude to the following individuals who played a key role in bringing this conference to fruition.

**CFRI Professional and Volunteer of the Year Awards Panel**
- Francine Bion
- Barbara Curry
- Colleen Dunn, MS, RT, CCRD
- Oscar Flamenco, CPA
- Marina Gonzales
- Danielle Mandella
- John Mark, MD
- Carole Nakamura, RN
- Yelizaveta Sher, MD
- Ahmet Uluer, DO

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

**Awards Celebration Special Guests**

**Will Battersby — Director of Salt In My Soul**
Will Battersby is a partner at Reno Productions, a New York film, television and theatre production company that owns and operates the Westside Theatre. He has been making documentaries and features for 15 years. His credits include *Trumbo*, which won the National Board of Review Freedom of Speech award; *The Spine of Night*; *Stephen King’s A Good Marriage*; the Oscar-nominated documentary *Enron: The Smartest Guys in the Room*; and *They Remain*. His films have played at major international film festivals such as Toronto and SXSW and been released worldwide. *Salt in My Soul*, Will's second documentary as director, was released worldwide in January 2022 to universal acclaim. *The Canal*, which he is producing and directing with Patricia Arquette, will be released by Showtime, which is currently adapting it into a series.

**Emily Schaller — Executive Director of the Rock CF Foundation**
Emily Schaller, 40, is a heroine with one goal in mind - to Rock CF. Equal parts spark, wit and humor, Emily is claiming her victories against cystic fibrosis having launched the Rock CF Foundation in 2007 to heighten public awareness and raise funds to increase the quality of life for everyone with CF. Emily created and manages an internationally acclaimed line of merchandise to help fulfill the mission of Rock CF. Today, Emily’s battle against this deadly genetic disease is printed in *Runner’s World, FORBES, The Atlantic and SPIN* magazines, the New York Times, The Washington Post, USA Today, NPR the Associated Press, and various cystic fibrosis focused educational websites. She is a marathon running super teacher and speaker, addressing parents, patients and audiences about the effects of cystic fibrosis and the ever changing and improving treatments being made. Through Emily’s humor and personal experience, she inspires the masses to transform their lives with exercise, diet and goal setting.
Special Welcoming Remarks

Janet Woodcock, MD
— Principal Deputy Commissioner, US Food and Drug Administration

Dr. Woodcock serves as the FDA’s Principal Deputy Commissioner, and was the Acting Commissioner from January 2021 until February 2022. Dr. Woodcock is board certified in internal medicine. She holds a Doctor of Medicine from the Feinberg School of Medicine at Northwestern University Medical School and completed a fellowship in rheumatology. Dr. Woodcock began her FDA career in 1986 at the Center for Biologics Evaluation and Research (CBER). She later became Director of CBER’s Office of Therapeutics Research and Review, which during her tenure oversaw the approval of the first biotechnology-based treatments for multiple sclerosis and cystic fibrosis.

In 1994, Dr. Woodcock was named Director of the FDA’s Center for Drug Evaluation and Research (CDER), where she implemented many of the FDA’s drug initiatives. In 2004, Dr. Woodcock became the FDA’s Deputy Commissioner and Chief Medical Officer, and later served in other executive leadership positions in the Commissioner’s Office. In 2007, Dr. Woodcock returned as Director of CDER until selected to serve as the therapeutics lead for “Operation Warp Speed” in 2020 to support the development, evaluation, and availability of treatments such as monoclonal antibodies and antiviral drugs for patients with COVID-19.

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 of the Bay Area. Bill is currently in his fifth two-year term serving on the West Valley/Mission College Citizens Bond Oversight Committee, and has served for the past ten 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, and hiking.

CFRI Executive Director — Siri Vaeth, MSW

Siri Vaeth has been CFRI’s executive director since 2018, but her involvement with the organization began soon after her daughter Tess’ diagnosis with CF in 1995. As a CFRI volunteer, she raised funds, chaired the Newsletter Committee, and served for 10 years on the Board of Directors. She joined CFRI’s staff in late 2013. Siri has a BA in Politics from UC Santa Cruz, and a Master’s in Social Welfare from UC Berkeley. She brings many years of nonprofit experience to CFRI, previously serving as executive director of Big Brothers Big Sisters of Santa Cruz County, nonprofit grant writer and consultant, United Way campaign associate, and social worker with Migrant Head Start. In addition to Tess, Siri has a 23-year-old son. She lives in Santa Cruz, California.

Master of Ceremonies — Jim Hampton

Jim Hampton is the father to twin 29-year-old daughters with cystic fibrosis and a 25-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. Along with his wife, Marilyn, Jim has been involved with CFRI for many years, and he is a former member of the Board of Directors.
Donald L Chi, DDS, PhD
University of Washington / Seattle, WA

Donald L. Chi is a Professor and the Lloyd and Kay Chapman Endowed Chair of Oral Health at the University of Washington School of Dentistry (Seattle, WA) and serves as Associate Chair for Research in the Department of Health Systems and Population Health in the School of Public Health. He is dual board-certified in pediatric dentistry and dental public health. Chi’s research focuses on understanding and addressing oral health inequities. He received the 2017 IADR Distinguished Scientist Award and was named Pediatric Dentist of the Year by the American Academy of Pediatric Dentistry in 2018. He spent the 2016-2017 academic year as a Fellow at the Center for Advanced Study in the Behavioral Sciences at Stanford University. Chi teaches public health to dental students and pediatric dentistry residents and has been a staff pediatric dentist at the Odessa Brown Children’s Clinic in Seattle since 2009.

Jacob Fraker, MSW
Office of California State Senator Susan Eggman / Sacramento, CA

Jacob Fraker was born and raised in the small rural town of Sonora, CA. Jacob was diagnosed in utero with cystic fibrosis after his older brother was diagnosed at age 3 due to salty sweat. Jacob used to travel 3 hours from Sonora to San Francisco to get care until moving to San Jose for his undergraduate studies. Jacob received his Bachelor’s in Social Work at San Jose State University. During his time there, he served as the President of the Undergraduate Social Work Association and worked at the Homeless service center in Santa Cruz and at UCSF Citywide in the Tenderloin of SF. In 2017 following a legislative fellowship, Jacob began working with CFRI on rare disease policy issues. Jacob then went on to UC Berkeley where he got his Master’s in Social Welfare in their Strengthening Organizations and Communities track. During his time at UC Berkeley, Jacob interned with CFRI helping establish a rare disease presence in the State Capitol and then later went on to intern in the office of Assemblymember Susan Eggman. Jacob carried bills, staffed the member in policy committee, and advised on health policy. Following his graduation, Jacob worked at the Governor’s Office of Emergency Services as a Health and Housing Recovery Specialist before returning to the Capitol to join now Senator Eggman’s team as the Senate Consultant to the LGBTQ Caucus. In this role, Jacob advises 8 state legislators on broad issues relating to the LGBTQ+ Community. He also works as a policy analyst for the Senator carrying bills and advising on military and veteran affairs, human services, insurance, and rare disease issues.

Christine Hachem, MD
Saint Louis University / Saint Louis, MO

Christine Hachem is a Professor of Medicine in the Division of Gastroenterology at Saint Louis University. As a faculty member, she has taken a strong interest in the clinical care of individuals with cystic fibrosis and education of colleagues and the community about the topics of chronic gastrointestinal issues such as dysmotility, acid reflux, and colon cancer screening in cystic fibrosis. She has engaged in both public, patient/family, and resident/student education on the importance of gastroenterology topics for the CF community with a focus on colon cancer screening in individuals with CF. She has served on numerous local, regional and

Continued on page 14
Speaker Profiles

Continued from page 13

national committees committed to the education and quality of care for patients. She is the governor of the American College of Gastroenterology society in Missouri. She is also currently the principal investigator of a multicentered study, in conjunction with the Cystic Fibrosis Foundation, looking at stool-based colorectal cancer screening options in individuals with CF.

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

Dr. Jain is a Professor of Medicine and Pediatrics at Northwestern University’s Feinberg School of Medicine. He graduated from Northwestern University Phi Beta Kappa and with Highest Distinction with a Bachelor of Arts degree in Biochemistry. He received his MD from the University of Chicago and completed his residency in Internal Medicine and fellowship in Pulmonary Critical Care also at the University of Chicago. He joined the faculty at Northwestern in 1996 and has risen through the ranks as a full professor. He has received funding from the NIH, Veterans’ Administration and CF Foundation for his investigator-initiated research. He has been the Director of the Adult CF Program at Northwestern since 1997.

He has published more than 100 peer-reviewed articles and has been on numerous national CF boards and committees. Currently he is the adult program representative on the CF Foundation’s Center Committee and the Planning Committee for the North American Cystic Fibrosis Conference. In addition, he has been on the Therapeutics Development Network (TDN) steering committee and has been the past Chairman of the Protocol Review Committee for the TDN. He is presently the co-chair of the Guidelines Steering Committee and Genetic Therapies Working Group for the CFF. He is on the editorial board for AJRCCM and has been a reviewer for CHEST, Thorax, EMBO Molecular Medicine, Journal of CF, Translational Research among other journals. He has also served as an ad-hoc reviewer for numerous NIH panels and CFF and CFF Canada.

Emily Kramer-Golinkoff, MBE
Emily’s Entourage / Merion Station, PA

Emily Kramer-Golinkoff is Co-Founder of Emily’s Entourage, an innovative 501(c)3 foundation that accelerates research for new treatments and a cure for nonsense mutations of cystic fibrosis. She is also an internationally recognized patient advocate and speaker.

Emily’s Entourage has awarded millions of dollars in research grants since 2011, launched a CF gene therapy company acquired by a large pharmaceutical company, developed a CF nonsense mutation patient registry and clinical trial match-making program to accelerate clinical trial recruitment, and led worldwide efforts to drive high-impact research and drug development. The organization has been featured in national media outlets, including New York Times, STAT, CNN, Yahoo, AOL, People, The Philadelphia Inquirer and more.

Emily has a Master’s degree in Bioethics and certification in clinical ethics mediation from the University of Pennsylvania, where she also completed her undergraduate degree. She has given talks at the White House, TEDx, University of Pennsylvania’s Annenberg School for Communication Commencement, Stanford University’s Medicine X Conference, and more. Emily was named a “Champion of Change” for President Obama’s Precision Medicine Initiative and is the recipient of the 2020 Philadelphia Magazine Luminary Award and the 2016 Global Genes Rare Champion of Hope for Advocacy Award.
Speaker Profiles

Paul B. McCray, Jr., MD
University of Iowa / Iowa City, IA

Dr. Paul McCray is a Professor of Pediatrics, Division of Pulmonary Medicine, and Professor of Microbiology and Immunology. His scientific interests include cystic fibrosis, epithelial cell biology, innate immunity, host-pathogen interactions, and the applications of gene transfer for lung diseases. His team used molecular, genomics, and bioinformatic approaches to discover novel secreted peptides and proteins with host defense functions in airway epithelia. He is investigating gene addition approaches for CF using integrating viral vectors, and more recently has applied this knowledge to investigate delivery of gene editing tools to the respiratory tract. Coronavirus projects in his lab aim to develop a better understanding of the pathogenesis of severe coronavirus pulmonary infections, including SARS, MERS, and COVID-19. The McCray lab has helped develop and characterize mouse models of SARS, MERS-CoV, and more recently COVID-19, and is using these models to study new treatments and preventions. He is an elected member of ASCI, AAP, AAAS, and AAM. Dr. McCray’s work is supported by the NIH and the Cystic Fibrosis Foundation.

Dao Nguyen, MD.CM, FRCP(C)
McGill University / Montreal, Canada

Dr. Dao Nguyen is Associate Professor of Medicine at McGill, Clinician Scientist at the Research Institute of the McGill University Health Centre, Physician in the Division of Respirology at the McGill University Health Centre and Founding Director of the McGill Antimicrobial Resistance Centre. Her research program is focused on the pathogenesis of cystic fibrosis lung infections, the mechanisms of antibiotic tolerance and host-pathogen interactions of Pseudomonas aeruginosa.

She has been a recipient of numerous prestigious career awards, including the Fonds de Recherche Sante Quebec Clinical Chercheur Boursier, CIHR clinician scientist, Burroughs Wellcome Fund Career Award for Medical Scientist, Vertex Cystic Fibrosis Research Innovation and Cystic Fibrosis Canada Scholar awards. She has published over 50 peer-reviewed publications (including in journals such as Science, PNAS, Science Advances, Am J Respir Crit Care Med), and has given more than 50 invited talks at national and international meetings and seminars. Her work has been consistently funded by national and international sources including CIHR, NSERC, Cystic Fibrosis Canada, Cystic Fibrosis Foundation, CFI, Burroughs Welcome Fund, the Department of Defense, and the NIH.

Hanna Phan, PharmD, FCCP, FPPA
University of Michigan / Ann Arbor, MI

Hanna Phan, PharmD, FCCP, FPPA is Clinical Associate Professor in the Department of Clinical Pharmacy, College of Pharmacy and Faculty Affiliate for the Susan B. Meister Child Health Evaluation and Research (CHEAR) Center at the University of Michigan. Dr. Phan practices as a Clinical Pharmacist Specialist in Pediatric Pulmonary Medicine with a focus in cystic fibrosis (CF), at C.S. Mott Children’s Hospital, Michigan Medicine. Prior to her return to Michigan, she served as faculty at the University of Arizona Colleges of Pharmacy and Medicine as well as Associate Research Scientist for the UA Asthma and Airway Disease Research Center. Dr. Phan received her Doctor of Pharmacy (PharmD) from the University of Michigan and

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Speaker Profiles

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completed a Postdoctoral Fellowship in Pediatric Pharmacotherapy at the Ohio State University. She has been recognized for her contributions to clinical practice, service, and research from various national professional organizations, including having previously served as President of the Pediatric Pharmacy Association. Dr. Phan's current research interests include patient-caregiver education and medication adherence, medication use in schools, and interprofessional care of children with chronic illness such as cystic fibrosis and asthma.

Joseph M. Pilewski, MD
University of Pittsburgh / Pittsburgh, PA

Dr. Joseph M. Pilewski is an Associate Professor of Medicine, Pediatrics, and Clinical and Translational Science at the University of Pittsburgh and University of Pittsburgh Medical Center. He is Associate Chief for Clinical Affairs in the Pulmonary, Allergy, and Critical Care Medicine Division at UPMC, and was Medical Director of the Lung Transplant Program at UPMC from 2004 to 2015 and 2021-2022. He is Co-director of the Cystic Fibrosis Program. He has been a CF and lung transplant physician for over 20 years. He has a long history of basic and clinical investigations related to cystic fibrosis and lung transplantation. His basic science studies focused on airway epithelial ion transport in primary human airway epithelial cells. His clinical investigations have focused on new therapies for CF, as he has been director of a CF Translational Studies Core and the principal or associate investigator on over 20 investigator-initiated and multi-center clinical trials in CF. He is an active contributor to the CF Therapeutics Development Network. As Medical Director of the Lung Transplant program, he directed clinical investigations focused on novel immunosuppressive regimens and improving our understanding of lung transplant candidate selection and complications. As co-Executive Director of the CF Lung Transplant Initiative, he is leading research efforts to improve the care of individuals with CF before and after lung transplantation.

Melissa S. Putman, MD, MMSc
Massachusetts General Hospital and Boston Children’s Hospital / Boston, MA

Dr. Putman is an adult and pediatric endocrinologist at Boston Children’s Hospital (BCH) and Massachusetts General Hospital (MGH) and an Assistant Professor at Harvard Medical School. Her interests include pediatric bone health, cystic fibrosis related bone disease, the prevention of adult endocrine complications of childhood diseases, and optimizing patient transitions from pediatric to adult care. She cares for children and adults with cystic fibrosis (CF) alongside their pulmonologists and other providers in the BCH CF Center and the MGH Adult CF Program. Funded by the NIH and Cystic Fibrosis Foundation, she performs clinical research focused on the endocrine complications of CF including CF-related diabetes and bone disease.
Diane Shader Smith
Los Angeles, CA

Diane Shader Smith is a writer, speaker, publicist, cystic fibrosis fundraiser, and advocate for bioethics, phage therapy, and AMR. 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, which were published posthumously as *Salt in My Soul: An Unfinished Life* by Random House. Diane has traveled the country speaking about her daughter’s writing, sharing profound insights about the hardships of living with chronic illness, the patient experience, organ transplant, phage therapy and antimicrobial resistance (AMR), among other topics. In early 2022, 3Arts Entertainment produced a documentary film, *Salt in My Soul*, based on Mallory’s memoir, which has provided another platform to share Mallory’s insights.

Richard H. Simon, MD
University of Michigan / Ann Arbor, MI

Dr. Simon graduated from Duke University Medical School in 1972 and then completed a two-year residency in the Department of Medicine at the University of California San Francisco. He was next a research associate in the Laboratory of Molecular Biology at the National Institute of Arthritis, Metabolic, and Digestive Diseases in Bethesda, MD and then moved to the University of Colorado to serve as chief medical resident followed by a fellowship in Pulmonary and Critical Care Medicine. In 1981, Dr. Simon joined the faculty of the Division of Pulmonary & Critical Care Medicine at the University of Michigan where he served for 40 years before becoming an emeritus professor in 2021. While a pulmonary fellow in Colorado, Dr. Simon assisted in the care for what was then only a small number of adults with cystic fibrosis. This early involvement led to one of his major career focuses and allowed him to contribute to the amazing progress in survival and wellbeing of people with CF. He serves on multiple committees for the Cystic Fibrosis Foundation including the Medical Advisory Council, Data Safety Monitoring Board, Clinical Research Executive Committee, and Clinical Research Advisory Board. He was the recipient of the Richard C. Talamo Distinguished Clinical Achievement Award from the Cystic Fibrosis Foundation in 2021.

Steffanie Strathdee, PhD
University of California San Diego / San Diego, CA

Steffanie Strathdee, an infectious disease epidemiologist, is Associate Dean of Global Health Sciences and Harold Simon Distinguished Professor of Medicine at the University of California San Diego where she codirects the Center for Innovative Phage Applications and Therapeutics (IPATH). In 2016, Strathdee and colleagues were credited with saving her husband’s life from a deadly superbug infection using bacteriophages – viruses that attack bacteria. The case, which involved cooperation from three universities, the U.S. Navy and researchers across the globe, shows how phage therapy is a future weapon against multi-drug resistant bacterial infections which are expected to kill 10 million people per year by 2050. Strathdee and Patterson co-authored a book on their story called *The Perfect Predator: A Scientist’s Race to Save Her Husband from a Deadly Superbug*. For her efforts to revitalize phage therapy in the West, she was named one of TIME magazine’s Most Influential People in Health Care in 2018.
Stephen G. Aller, PhD
University of Alabama at Birmingham / Birmingham, AL

Dr. Stephen Aller is an Associate Professor at the Department of Pharmacology and Toxicology at the University of Birmingham in Alabama. In 1988, he studied regulation, molecular biology, and electrophysiology of CFTR from the shark Squalus acanthias compared to the human channel as a Hearst Foundation High School Research Scholar at the Mount Desert Island Biological Laboratory (Bar Harbor, Maine). In his Master’s Degree (M.S.) thesis research, he cloned and characterized the first guanylyl cyclase receptor (NPR-B) and showed activation of CFTR-dependent chloride conductance through cGMP-mediated phosphorylation. As part of his Ph.D. thesis research, he received training in expression, purification and protein crystallization, and solved the structure of a human metal uptake transporter by cryo-electron microscopy (cryo-EM). This work led to an NIH-postdoctoral fellowship at the Scripps Research Institute, where he established overexpression, purification, and crystallization protocols and solved the X-ray crystal structure of the mammalian ABC protein P-glycoprotein (ABCB1), a homolog of CFTR (ABCC7). His lab is currently studying the structure-function of CFTR by single-particle cryo-EM. He established key collaborations at UAB in this regard, including work with Drs. Steven Rowe and Wei Wang to challenge the currently accepted binding location of potentiator drugs to CFTR by patch clamp and cryo-EM. He is also a major user on UAB’s newly installed ThermoFisher Glacios 200kV electron microscope for cryo-EM which was funded by a successful NIH S10 Award (S10OD024978) to which he contributed major grant writing and a hands-on 3-day in-person data processing workshop held on the UAB campus. He has two extremely talented trainees in the laboratory who have contributed the micrographs and classification of CFTR R933 mutants for the 2022 CFRI Conference.

Gregory Burkeen, PhD Candidate
San Diego State University / San Diego, CA

Gregory Burkeen completed his undergraduate education in 2013, with a major in international relations at Florida International University in Miami. He attended with a full ROTC scholarship and received an active duty Army commission from the same institution upon graduation. Gregory started pursuing science in 2015 while on deployment as an advisor to the Ukrainian army. The result of that was a white paper on how to use celestial geodesy/navigation to overcome the issues associated with the Russian military jamming the signals from Global Positioning Satellites in Donetsk and Luhansk.

Gregory has been enrolled as a graduate student at SDSU’s cell and molecular biology department since 2018 and has been working as a PhD student in Drs. Anca Segall and Forest Rohwer’s labs since April 2020. His current studies focus on phage therapy for cystic fibrosis patients enrolled in clinical trials at UCSD. He assesses the efficacy of phage therapy via metagenomics and bacterial culture. He is also working on the production of new theoretical and in vitro models to study hypothetical therapies for CF lung disease as a part of his doctoral dissertation.
CFRI-Funded Researcher Profiles

Steven J. Jonas, MD, PhD
University of California Los Angeles / Los Angeles, CA

Dr. Steven J. Jonas is a pediatric physician scientist and Assistant Professor in the Department of Pediatrics at the UCLA David Geffen School of Medicine and California NanoSystems Institute. He is a member of the UCLA Eli & Edythe Broad Center of Regenerative Medicine & Stem Cell Research and the Jonsson Comprehensive Cancer Center. His multidisciplinary research team targets the development and application of new technologies and methods to support the children’s health and regenerative medicine research communities in accelerating the discovery and implementation of emerging gene & cellular therapeutic approaches and precision medicine-based diagnostic tools. A primary focus of this research explores strategies for improving how gene therapies are manufactured through the design of nanotechnologies that enable rapid, safe, cost-effective, and efficient delivery of genes and genome-editing machinery. These capabilities motivate the Jonas group’s efforts to create tools that enable stem cell biologists to probe and to interact with stem cells more precisely and empower clinical scientists to apply this knowledge to design and implement new therapies more rapidly and broadly.

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

Naren Gajenthra Kumar, BTech, PhD
University of California Berkeley / Berkeley, CA

Since his early days, Dr. Naren G. Kumar has been interested in how environmental stimuli influence bacterial adaptation in diverse environments. Dr. Kumar received his Bachelors in Biotechnology Engineering from SRM University, Kattankulathur, India in 2014. He moved to Virginia Commonwealth University in Richmond Virginia to pursue his PhD in Microbiology and Immunology under the joint mentorship of Drs. Kimberly K Jefferson and Dayanjan S Wijesinghe. In his doctoral work, he applied mass spectrometry-based lipidomic analysis to the study of bacterial lipid-modifying enzymes and their role in bacterial biofilm formation, response to oxidative stress, and wound healing. Using the pathogen Staphylococcus aureus as a model system, his doctoral thesis focused on understanding the interplay between bacterial infections and the lipid-mediated host response.

Having witnessed his father experience a life-threatening recurrent pseudomonas infection that was nonresponsive to antibiotic treatment, Dr. Kumar joined the lab of Dr. Suzanne Fleiszig at UC Berkeley in the spring of 2020 as a postdoctoral research scholar to study the lifestyle of intracellular Pseudomonas aeruginosa and the etiology of microbial keratitis. Here, his research focuses on understanding how the host environment contributes to the evolution of bacterial pathogens that evade antimicrobial treatment and the host immune response. His ongoing work combines high-resolution imaging methods with omics-based technologies to understand the adaptation of intracellular Pseudomonas aeruginosa. Using this approach, his research aims to identify novel therapeutic targets to better manage challenging bacterial infections.
CFRI-Funded Researcher Profiles

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 received 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 six 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.

Dr. Li’s main research objective is to investigate the roles of the distal small airways in the pathogenesis of cystic fibrosis (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, while 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. 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.

Sriram Vaidyanathan, PhD
*Stanford University / Palo Alto, CA*

Dr. Sriram Vaidyanathan was born in Thiruvananthapuram, India. He graduated from Purdue University in 2010 with a B.S.E. in Biomedical Engineering. He joined the Banaszak Holl group at the University of Michigan in 2011 and obtained an M.S.E in Biomedical Engineering in 2013. As a doctoral student, he studied the intracellular transport of non-viral gene delivery systems to optimize gene delivery. He completed his PhD in 2016 and joined the Porteus lab at Stanford University to further his interest in applying CRISPR/Cas9 based methods to develop therapies for monogenic diseases. As a postdoctoral scholar, he has spearheaded a multi-disciplinary team from four different labs at Stanford to develop an autologous airway stem cell therapy for cystic fibrosis. A recent publication from this team described the high efficiency correction of the F508del mutation in airway stem cells for the first time. He is currently working on extending this platform to develop a genome editing platform that restores CFTR function in airway cells affected by any CF mutation, including those that cannot be treated using modulators. In the future, his goal is to develop methods to transplant corrected stem cells back into the airway and thus enable the development of a durable therapy for CF.
Saima Aslam, MBBS, MS  
*University of California San Diego / San Diego, CA*

Dr. Aslam is a Professor of Medicine at the Division of Infectious Diseases and Global Public Health at the University of California San Diego (UCSD). She is a transplant infectious diseases physician and is the Director of the Solid Organ Transplant Infectious Diseases service at UCSD. She has been engaged in phage therapy since 2017 and is the Clinical Lead at the Center for Innovative Phage Applications and Therapeutics (IPATH) at UCSD. Dr. Aslam currently has funding through the Cystic Fibrosis Foundation for a pilot study to develop a clinical registry of *Burkholderia* infected patients with CF and develop an associated bacteriophage library. She is also co-investigator in a U01 grant from NIH/ NIAID to combat multi-drug resistance through innovative applications, including phage therapy. Dr. Aslam is involved in multiple transplant-related clinical trials as well as an ongoing study investigating the use of phage-lysin for *Staphylococcus aureus* bacteremia.

Ella Balasa, BS  
*Richmond, VA*

Ella Balasa is a patient advocate, consultant, and a person living with cystic fibrosis. She was diagnosed at 18 months old and has experienced countless hospitalizations since being a child. She has committed her time to empowering patients and advancing research and healthcare strategies through her connections with researchers, pharmaceutical companies, and patient organizations. She is an advocate for the development of novel therapies for the treatment of antibiotic-resistant infections and speaks publicly at conferences, meetings, and to companies about the value of the patient perspective. She also has a passion for writing, distilling clinical information for patient communities and sharing about the hardships and triumphs that come with living with a chronic illness. She has been published in journals, news sites, and blogs. Through opportunities working with healthcare organizations and sharing her journey through content strategy, writing, public speaking, clinical trial development, and sharing the patient experience, she aims to affect the healthcare landscape by raising awareness of rare diseases, promoting self-advocacy to patients, and valuable insights to organizations. More of her work and experiences can be found at [www.ellabalasa.com](http://www.ellabalasa.com).

Marina Gonzales  
*San Jose, CA*

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, including legislative visits during CFRI’s CF Awareness events in May. She also serves on CFRI’s Gala Committee. Marina is the Marketing Manager at San Francisco’s Ghirardelli Chocolate Company, and lives in San Jose California.
Panelist Profiles

**Suraj Patel**  
San Antonio, TX

Suraj Patel is a Software Test Engineer for Accenture Federal Services. He is 29 years old and lives in San Antonio, Texas. Suraj has one dog named Kaya. In his free time, he enjoys hiking, cooking, and playing video games. Suraj has a blue belt in Brazilian Jiu-Jitsu, and has been training and competing in this martial art for 4 years. A life goal of his is to get his black belt in Brazilian Jiu-Jitsu and be able to help and teach others in hopes it changes their lives the way it has changed his life.

Suraj is the brother of an adult with cystic fibrosis. He was 13 years old when his brother was diagnosed with CF. Since that time, he has seen his brother go through numerous hospital visits and long-term hospital stays. He has supported his brother and learned from his condition the importance of taking care of one’s own health and body. Learning the limitations of someone who has a lung disease has shown him how important it is to exercise and train one’s lungs every day to be able to breathe better by doing cardiovascular exercises and breathing exercises. His brother’s diagnosis has helped him appreciate the importance of health and understand the struggle others have to go through when dealing with conditions like CF.

**Thomas Patterson, PhD**  
San Diego, CA

Thomas Patterson is Distinguished Professor of Psychiatry at the University of California San Diego School of Medicine. He is an experimental psychologist with research interests in stress and its impact on health, measurement of functioning in cognitively impaired individuals, and developing interventions focused on HIV prevention in high-risk populations. He has conducted research in a number of areas including HIV/AIDS prevention, rehabilitation of older patients with psychosis, and stress responses of caregivers of Alzheimer’s disease patients. He has authored or co-authored over 400 scholarly papers, 17 book chapters, and a widely used textbook on human behavior. He was a founding editor of the journal AIDS and Behavior and has served as co-editor and on the editorial boards of a number of other journals. In March 2016, Dr. Patterson became the first known person in the United States to successfully undergo intravenous bacteriophage (phage) therapy.

**Damian Rice**  
Dacula, GA

Damian Rice is a native of Oakland, California. He currently lives in Dacula, Georgia, which is a suburb of Atlanta. Cystic fibrosis has been a major part of Damian’s life ever since his twin brother, Everett, was diagnosed with the disease at age 9. Due to living miles apart and air quality issues, the two brothers only get to spend time together in California where the weather is less harsh for people living with CF. One thing they are both thankful for, though, is that they’ve shared 51 birthdays – an achievement that was not predicted when they were little. Damian continues to be amazed and inspired by his brother’s resilience and the advancements in research related to treating and managing CF.
Support / Discussion Group Facilitators

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

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, promoting CFRI’s counseling and Quality of Life services, and moderating a COVID-19 CF support group. In 2021, Dr. Sher was named CFRI’s CF Professional of the Year.

Ann Steiner, PhD
Dr. Steiner is a nationally-recognized psychotherapist, trainer, consultant, and author with a private practice in Lafayette. Since giving a keynote address for CFRI in 2000 she has returned every year to lead and to facilitate support groups. A dynamic and powerful speaker for over 25 years, she has presented throughout the U.S. and internationally for medical and mental health organizations, consumer groups and other illness-related organizations. She is passionate about the healing power of group work and is a Fellow of the American Group Psychotherapy Association, helped found and consults for the Group Therapy Training Program for The Psychotherapy Institute in Berkeley, and serves on the board of The International Human Learning Resources Network. Her book, Help Your Group Thrive: Workbook and Planning Guide for leaders of work groups, writer’s groups, book clubs, community and networking groups, was released in 2018. Drawing on her personal and professional experience with chronic conditions, Dr. Steiner produced a CD, The Rollercoaster of Chronic Illness: How to Add Joy to the Ride, which is also the title of her soon to be published self-help book.
Support / Discussion Group Facilitators

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

Panel Moderators

Julie Desch, MD
At 61 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.

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

Antimicrobial resistance is one of the most pressing global health issues of the 21st century and is a growing health concern among patients living with cystic fibrosis (CF). With existing antibiotics losing potency and no new classes of antibiotics in the pipeline, alternatives to antibiotics are urgently needed. In 2016, Dr. Strathdee was involved in a remarkable case where she and her colleagues revived a hundred-year-old forgotten cure – phage therapy - which saved her husband’s life from a deadly superbug infection. Since then, UC San Diego faculty have used phage therapy to successfully treat multi-drug resistant bacterial infections in dozens of other cases and consulted on dozens more internationally, including the first use of a genetically modified phage cocktail to treat a systemic Mycobacterium abscessus infection in a CF patient. In 2018, UCSD’s Chancellor provided seed funding to launch the Center for Innovative Phage Applications and Therapeutics (IPATH), the first dedicated phage therapy center in North America. Several clinical trials of phage therapy are now underway, with phage therapy being regarded as one of the most promising alternative and adjunct to antibiotics. Strathdee will share the details of her story, which was the subject of a memoir she wrote with her husband, The Perfect Predator: A Scientist’s Race to Save her Husband from a Deadly Superbug, as well as challenges and future prospects for phage therapy for CF patients.

CF Airway Infections: To Treat or Not To Treat? Why and How?  
Dao Nguyen, MD,CM, FRCP(C)  
McGill University, Montreal, Canada

The airways of people with CF become infected with different microbes, particularly bacteria, and antibiotics are a major part of the treatment regimens. While some infections lead to increased respiratory symptoms and more rapid decline in lung function, the consequences of other microbes in the CF airway are less clear. How do we diagnose airway infections? Which microbes cause disease? How do we determine whether to treat and what the treatments of choice are? In this presentation, I will review the recent advances in our understanding, diagnosis and treatment of CF airway infections, as well as the challenges and remaining questions.

Understanding Gastrointestinal Cancers in Cystic Fibrosis  
Christine Hachem, MD  
Saint Louis University, Saint Louis, MO

People with cystic fibrosis (CF) are living longer and we are seeing an adult CF population increase. The median survival age is now 50 years and has increased by 10 years in the last 2 decades. However, with improvements in therapies for CF, we are also seeing an increase in GI cancers as CF is a multisystem disease that involves the GI tract. Multiple observational studies have demonstrated an increased risk of GI cancers. Some propose that cystic fibrosis is in fact a GI cancer syndrome. The pathogenesis of increased GI cancer risk in CF is unclear but multiple studies demonstrate impairments in CFTR and increased viscosity of secretions leading to chronic inflammation, bacterial dysbiosis and upregulation of oncogenic genes. Indeed, CFTR is a tumor suppressor gene that is downregulated in GI cancers. Multiple risk factors play a potential role in the pathogenesis of GI cancers in CF. GI cancers in cystic fibrosis appear at younger ages. In addition, diagnosis is delayed and difficult because of the high prevalence of GI symptoms in CF.

The most common GI cancer in cystic fibrosis is colorectal cancer (CRC). Nearly all CRC diagnosed in CF is before the age of 50. Screening for CRC in CF should start at age 40 if no history of transplant and age 30 if history of transplant as transplant history increases risk of CRC 30-fold. Colonoscopy is the only recommended screening test for CRC in CF. However, a multicenter trial is evaluating stool-based
screening for CRC. Multiple screening algorithms for other GI cancers in CF have been suggested but the only clear recommendations for GI screening in CF are in CRC at this time. Most GI screening guidelines recommend starting at age 40 or immediately after transplant as prevention and early detection are likely the key to preventing the continued rise of GI cancers in CF. The epidemiology, etiology and risk factors for GI cancers in CF will be discussed. Screening recommendations for GI cancers in CF will be reviewed.

Advances in Gene Therapy for Cystic Fibrosis

Paul B. McCray, Jr., MD
University of Iowa, Iowa City, IA

Remarkable progress in our understanding of CF pathogenesis has occurred in recent years, yet the disease remains progressive with significant morbidity and mortality. Advances in knowledge of CFTR biogenesis and function led to the development of small molecule modulator therapies that are helping many patients. However, ~10% of people with CF do not benefit from current CFTR modulators, including people with nonsense or splicing mutations and those intolerant of the medications. In addition, CFTR modulator therapies are life long and expensive. Thus, there is a continued interest in developing new therapies that address the needs of all people with CF. Although CF is a multi-organ system disease, most people with CF die of progressive lung disease that begins early in childhood and is characterized by chronic bacterial infection and inflammation. For this presentation, a focus will be on gene therapy for lung disease treatment and prevention.

Within a year of the discovery of the CFTR gene in 1989, the in vitro proof-of-concept for gene therapy for CF was quickly established. This indicates that a CFTR gene replacement approach would be efficacious regardless of the disease-causing mutation. In 1993, the first of many gene therapy clinical trials attempted to rescue the CF defect in airway epithelia. Despite the initial enthusiasm, there are no FDA-approved gene therapies for CF. This presentation will overview the history of CF gene therapy, from the discovery of the CFTR gene to newer gene delivery strategies and novel gene repair technologies.

In recent years, the field has witnessed significant advances in the development of new gene addition and gene editing strategies for the treatment of monogenic disorders. In this review, we will summarize current developments in gene therapy for cystic fibrosis. Central to all new therapeutic approaches is how to deliver the gene therapy tools. The epithelial cells lining the airways present many barriers, making delivery challenging. The airway environment of the chronically diseased lung presents additional barriers. We will explore the different gene addition and gene editing strategies under investigation and review the challenges of delivery to the lung. While implementation of CF gene therapy has proven more challenging than initially envisioned, thanks to continued innovation, it may yet become a reality.

The Challenges of Aging for People with CF

Richard H. Simon, MD
University of Michigan, Ann Arbor, MI

The survival of people with cystic fibrosis (CF) is rapidly increasing due to the development of highly effective CFTR modulators. As we all celebrate the benefits that these treatments have brought to many people with CF, we need to pay closer attention to several areas of health that were previously overshadowed by the more pressing problems caused by CF.

The health challenges of aging with CF obviously overlap with those conditions faced by all people such as decreasing mobility, reduced strength, osteoarthritis, and memory loss. But added to these are complications of CF that become more frequent as people age such as CF-related diabetes, CF-related bone disease, and gastrointestinal cancers. Fortunately, these conditions can be detected early and there are treatments that will lessen their consequences. For example, colon cancer, the most frequent form
of cancer in people with CF, can be prevented by appropriate screening and early treatment.

Another category of problems related to aging are the consequences of past or current CF treatments. A prime example is the side effects of antibiotics, particularly intravenous tobramycin. It is becoming more apparent that repeated courses over many years can lead in some people to progressive hearing loss, balance problems, and/or decreasing kidney function. Appropriately, the CF Foundation is starting a clinical trial to determine whether tobramycin is really needed as part of antibiotic regimens for pulmonary exacerbations in patients with pseudomonas.

Although we all celebrate the wide range of benefits caused by CFTR modulators, there are consequences that many of us did not foresee. It has been known that CF seems to protect against some conditions commonly seen in aging populations such as high blood pressure, high cholesterol levels, coronary artery disease, stroke, and obesity. But returning CFTR function toward normal may be increasing the prevalence of these common conditions. This is requiring us to rethink some basic principles of CF care, for example the recommendation to eat a high calorie, high fat, high salt diet. A primary care physician who is well versed in preventive medicine will be an even more important member of the CF care team.

None of the problems of aging should detract from the amazing benefits of modulator therapies. By implementing the appropriate screening programs and incorporating preventive and treatment measures that work in the general population, the full benefits of the CFTR modulators should be realizable.

mRNA therapy for People with Cystic Fibrosis: Next Steps

Manu Jain, MD, MSCI
Northwestern University, Chicago, IL

Over the last ten years, highly effective CFTR modulators (HEMT) have transformed the lives of many people living with CF because of their unprecedented clinical impact. However, there are still a substantial minority of PwCF who lack treatment options for the underlying cause of their disease because they do not qualify for HEMTs, have experienced adverse events from HEMT, or had a relatively small clinical effect. This includes people of color who disproportionately do not qualify for modulators because they have CFTR mutations that can’t be treated with approved HEMT.

One option for these PwCF and potentially all PwCF is mRNA therapy. mRNA therapy is a mutation agnostic treatment because it could work for any person with CF regardless of their CFTR mutation. The underlying principle of CF mRNA therapy is to administer normal mRNA which leads to the production of normal CFTR protein, thus bypassing each person’s CFTR mutation. There are a number of technical and practical hurdles that must be overcome for this therapy to become an approved treatment option.

Several companies are currently working in conjunction with the CF Foundation and the Therapeutics Development Network (TDN) to assess the safety and efficacy of mRNA treatment for PwCF. Dr. Jain will provide an update on the principles behind mRNA therapy, the hurdles it faces to become a viable treatment option, and the various programs in clinical development. As such, mRNA therapy represents an important next step in our journey towards CF, cure found.

Oral Health Considerations for Individuals with CF

Donald L. Chi, DDS, PhD
University of Washington, Seattle, WA

Oral health is an important but commonly overlooked contributor to overall health. In this presentation, Dr. Chi will summarize the available scientific data on the oral health of individuals with CF, present information on CF-specific risk factors and behaviors that can lead to poor oral health, provide a sneak peek of ongoing studies aimed at understanding the links between oral and overall health in CF, and provide attendees with tips on how to optimize oral health over the CF life course.
Partnership in CF Care: Communication and Collaboration When Considering Complementary Therapies
Hanna Phan, PharmD, FCCP, FPPA
University of Michigan, Ann Arbor, MI

Although effective advances in therapies for the treatment of cystic fibrosis have accelerated considerably in the last decade, not all people with CF (PCF) are yet eligible for treatments such as cystic fibrosis transmembrane conductance regulator (CFTR) modulators. With research ongoing, there remain gaps in CF treatment. As such some PCF may consider or use complementary and alternative therapy or medicines (CAM) to help address certain symptoms such as insomnia, constipation, anxiety, or overall health. CAM includes a wide range of therapies and can be categorized as nutritional (i.e., taken by mouth, a dietary or herbal supplement), physical (e.g., massage), psychological (e.g., mindfulness) or any combination thereof. Complementary is a treatment approach that is non-mainstream and used together with conventional medicine, whereas alternative is used in place of conventional medicine. A newer term, integrative health, combines conventional, complementary, and alternative therapies, with emphasis on multimodal interventions to treat the whole person versus a single condition or organ system.

Some commonly reported used CAM among PCF include probiotics, melatonin, omega-3 fatty acids, curcumin, as well as combination products that claim to improve lung health. A common misconception is that natural is equivalent to safe; however, for those products that are consumed, they may be processed by the body in similar pathways as conventional medicines and thus may pose risk of drug interaction(s) or effect organ systems such as the liver over time with use. Unfortunately, there is often a lack of data describing direct effect of a given CAM product with a given conventional therapy (i.e., medication) and/or condition (e.g., CF). As a result, historically, use of CAM has viewed by clinicians in a fashion that discouraged open communication between care teams and PCF and caregivers. Recent survey data show a likely continued lack of communication and partnership regarding CAM use, including shared decision making about use and monitoring. The quality and amount of safety and effectiveness data regarding CAM in the setting of CF are variable. Thus, it is important to evaluate and discuss available data, including how it applies for a given individual with regards to risk versus benefit, and if elected, determine measures to appropriately monitor therapy. This session will review some of the common reported CAM products’ safety and effectiveness data, considerations in evaluating and discussing available data of products, and recommended best practices of coproducing integrative care in CF.

CF-Related Bone Disease: Current Evidence and Future Directions
Melissa S. Putman, MD, MMSc
Massachusetts General Hospital and Boston Children’s Hospital, Boston, MA

As life expectancy continues to improve for people with CF, non-pulmonary complications are becoming increasingly prevalent, including CF-related bone disease (CFBD). Osteoporosis and fractures can lead to significant morbidity in people with CF, particularly rib and vertebral fractures. Multiple risk factors may contribute to compromised bone health in CF, including vitamin D deficiency, pancreatic insufficiency, malnutrition, inflammation, glucocorticoid treatment, pubertal delay and hypogonadism, and reduced weight bearing activity. Interestingly, studies suggest that CFTR is expressed in human bone cells and may play a direct role in the pathogenesis of CFBD by affecting bone formation and bone resorption. Screening for CFBD is recommended in all adults with CF and in children with risk factor for low bone density. Prevention and treatment of CFBD focus on nonpharmacologic interventions to optimize bone health, including adequate calcium intake, vitamin D supplementation for a 25-hydroxyvitamin D level above 30 ng/mL, weight bearing activity as tolerated, ensuring adequate nutritional status, and minimizing bone toxic medications as possible. The first-line pharmacologic treatment for CFBD are oral or intravenous bisphosphonates, which have been shown to improve bone density in children and adults with CF, though fracture outcomes with this treatment
are limited. Early data suggest that treatment with ivacaftor may improve bone density in adults with the G551D-CFTR mutation, and future studies are needed to understand the role of CFTR in the skeleton and to determine the long-term effect of CFTR modulators on bone health, particularly in the era of highly effective modulator therapy.

Advocacy in the CF Realm

Sunday, July 31, 11:20 am

Jacob Fraker, MSW, and Diane Shader Smith
Sacramento, CA, and Los Angeles, CA

Defined as “the act or process of supporting a cause or proposal,” advocacy has always played a key role in advancing cystic fibrosis research, raising awareness of the disease, and addressing policies and legislation that impede access to medical therapies and care. In the first presentation of this two-part session, Jacob Fraker, an adult with CF who serves in multiple roles in the state Capitol, including as a legislative aide to a California State Senator, will provide an overview of the many ways in which members of our CF community can participate in advocacy efforts individually, through CFRI, and within coalitions. He will address some of the key issues that are currently impacting the CF and rare disease community across the United States.

Part-two of the session will feature Diane Shader Smith, who took on the role of advocate after her daughter Mallory lost her battle with antibiotic-resistant pathogens. Her initial interest was in raising awareness and money for phage therapy research and clinical trials, but she has recently layered on the larger issue of antimicrobial resistance (AMR), which includes the need for better treatments. Diane does this through her daughter Mallory’s posthumously published memoir, Salt in My Soul, and now the documentary of the same name produced by 3Arts Entertainment. Diane employs the power of story to address audiences worldwide about this urgent global health threat. In her presentation, she will talk about the need for new antibiotics, antifungals, and phage therapy, and the groups and efforts that have mobilized to respond to this health crisis. Diane will share how CF community members can engage and advocate for passage of legislation that will advance AMR research and resources, such as the Pasteur Act, with emphasis on the power of our personal stories in raising awareness among decision makers.

Expanding Opportunities for Lung Transplant for Individuals with Cystic Fibrosis

Sunday, July 31, 12:45 pm

Joseph Pilewski, MD
University of Pittsburgh, Pittsburgh, PA

Lung transplantation provides a treatment option for many individuals with advanced lung disease due to cystic fibrosis. Since the first transplants for CF in the 1980s, survival has improved and the opportunity for transplant has expanded to include individuals with who previously were not considered candidates for transplant. Criteria to be a transplant candidate vary significantly among transplant programs, highlighting that engagement of more than one transplant program may be necessary. Individuals with highly resistant CF pathogens, malnutrition, osteoporosis, CF liver disease and other co-morbidities may be suitable candidates for lung transplant, or if needed, multi-organ transplant. The transplant process involves several phases, from discussion of prognosis and referral to a transplant center, to transplant evaluation, to listing, transplant surgery and care after transplant. While the availability of highly effective CFTR modulators for many individuals with CF has improved lung function and slowed progression to respiratory failure, early discussion and referral regarding transplant as a treatment option is critical to maximizing opportunity and optimizing patient and family experience. The decision to be evaluated for transplant and to list for transplant are distinct, and early referral may provide a treatment option that can be urgently executed if needed. Survival after transplant for CF is improving, to a median survival of approximately 10 years, and most transplant survivors enjoy significant improvement in quality of life.
Presentation Abstracts

The Power of Science, Community and Desperation to Speed Lifesaving Breakthroughs for the Final 10%
Emily Kramer-Golinkoff, MBE
Emily's Entourage, Merion Station, PA

Emily Kramer-Golinkoff is an adult with cystic fibrosis and the Co-Founder of Emily’s Entourage, an innovative 501(c)3 that accelerates research for new treatments and a cure for nonsense mutations of cystic fibrosis (CF).

In her talk, she describes her experience growing up with CF and learning that she is a part of the approximately 10% of the CF community that is ineligible for CFTR modulators, a heart-shattering realization amplified by the advanced state of her lung disease. Rather than become paralyzed by the bleakness of her future, Emily became supercharged - driven by the steadfast belief that nobody in the CF community should be left behind and the reality that time is of the essence for everyone in the CF community and for herself. In 2011, she co-founded Emily’s Entourage with her friends and family with the singular goal of accelerating research and drug development for those in the final 10% with a focus on CF nonsense mutations.

Her talk explores the impact of CFTR modulators on the CF community and her experience being part of the 10% left behind due to ineligible mutations. She discusses the promise of science and innovation, each individual’s power to create transformative change, and the unique solidarity and commitment of the whole CF community to create a better future for everyone with CF.

CFRI-Funded Research Abstracts

Role of CFTR Arginine-933 in Channel Function and Potentiator Binding and Efficacy
Stephen Aller, PhD
University of Alabama, Birmingham, AL

Cystic fibrosis (CF) is a disease which is caused by a defect in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR). CFTR is an inherently difficult protein to study because: 1) it is located in the cell membrane; 2) folding of CFTR from healthy people is inefficient compared to other membrane proteins; 3) many mutations in CFTR that cause disease fold extremely inefficiently, and 4) it is very difficult to achieve sufficient folded-functional protein for biochemical studies. Recent breakthroughs in a technique called cryo-electron microscopy (cryo-EM) have allowed researchers to examine the atomic structure of small quantities of purified CFTR in unprecedented detail. Most notably are the remarkable cryo-EM structures determined in 2019 of CFTR bound to two different potentiator drugs, one being the FDA-approved life-saving drug Ivacaftor (VX-770). An unexpected feature of the high-quality structures of CFTR in the presence of potentiators is that the chloride channel of CFTR is still in a closed conformation. The location of VX-770 binding on CFTR, exactly halfway through the cell membrane, is highly intriguing. The positively charged amino acid sidechain of Arginine 933 (Arg933) resides at the bilayer midpoint in an energetically costly low dielectric environment and forms a polar contact with VX-770. The Arg933/ VX-770 contact is theoretically enhanced by the low dielectric and would seem to be a dominant theme of potentiator binding and function, if not an outright requirement. We explored this hypothesis by examining several mutations of Arg933. All nine mutations tested formed “band C” and several were “superfolders” with respect to wild type CFTR “band C” levels. Loss of the positive charge significantly reduced basal activity of the CFTR channel in patch clamp studies. An increase in inward rectification was also observed for several mutants. VX-770 largely
stimulated channel activity of R933 mutants that showed low basal activity. CFTR-R933Y produced clearly recognizable particles by negative stain electron microscopy (NSEM) and paves the way for high-resolution structure determination of R933 mutations to determine the role of Arg933 itself in chloride conductance and the mode of potentiator binding in the absence of the positive charge at position 933.

Nanotechnologies to Enable Cystic Fibrosis Gene Therapies

Saturday, July 30, 2:45 pm
Steven J. Jonas, MD, PhD
University of California, Los Angeles, CA

The emergence of robust and reliable genome-editing tools, such as the CRISPR/Cas9 system, is rapidly paving the way for innovative gene therapies that promise definitive cures for genetic diseases. Cystic fibrosis (CF) represents an opportune target for these emerging treatments as the disease results from a single gene defect stemming from mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Previous efforts to establish gene therapeutic strategies for CF have so far been met by significant challenges. For example, effective targeting and delivery of gene editing reagents to long-lived and self-renewing airway basal stem cell (ABSC) populations for gene correction in the CF lung has been especially difficult because of their protected location within the respiratory epithelium. This presentation highlights our multidisciplinary team’s approach to overcome these obstacles through the design and testing of CRISPR/Cas9-based gene-editing cargoes for CFTR correction and nanoparticle carriers that can serve as nanoscale “shipping containers” capable of packaging these CRISPR/Cas9 payloads. These engineered nanocarriers can be delivered as aerosolized suspensions to enable inhalable gene therapy solutions for CF. To test this capability, vibrating mesh nebulizer devices are applied to generate nanoparticle-laden aerosol droplets that are directed to ABSCs supported on air liquid interface (ALI) culture models that mimic the epithelial structure and organization of the human airway. The suite of tools and methods developed here serve as building blocks for establishing CF gene therapy solutions that are poised to accelerate progress toward our collective goal to find a permanent cure for CF on the steepest gradient possible.

Transplantation of Gene Edited Upper Airway Basal Stem Cells in Immunocompromised Mice Using Fibrinogen Based Scaffolds to Treat CF Sinus Disease

Saturday, July 30, 3:50 pm
Sriram Vaidyanathan, PhD
Stanford University, Palo Alto, CA

Gene therapy approaches that restore CFTR function have the potential to treat all CF patients. However, delivery of the CFTR gene in vivo using viral and non-viral strategies have been unsuccessful. Airway stem cell therapies have been proposed as an alternative. However, cell therapies have been limited by concerns that conditioning regimens needed to make space for exogenous stem cells in the lung epithelium may be life threatening. Recognizing this, we propose to first transplant gene corrected autologous airway stem cells into the sinuses to treat CF sinus disease. We began by developing methods to genome edit upper airway basal stem cells (UABCs) from the nose and sinuses at high efficiencies. We recently reported the use CRISPR/Cas9 and adeno-associated viruses (AAV) to insert the full-length CFTR cDNA in the endogenous CFTR locus in airway basal stem cells. Epithelial sheets derived from corrected basal stem cells restore CFTR function to levels seen in non-CF controls. The transplantation of the corrected airway stem cells is the next technical hurdle that needs to be resolved for the clinical translation of this approach. In preliminary in vivo experiments, we discovered that the delivery of cells in saline into the nasal cavity of mice resulted in their expulsion. Therefore, we attempted to identify biomaterial scaffolds that will adhere to the tissue and facilitate the engraftment of the gene corrected airway stem cells. We evaluated the ability of several materials including type I collagen, laminin foam-gel, fibrinogen, alginate, hyaluronan and dextran to support the survival and proliferation of UABCs. We used basement membrane extract from mouse tumors (Matrigel™) as controls. Among the materials tested, UABCs seeded in laminin and fibrinogen showed 5±1 and 9±6-fold expansion respectively compared to 4±4 fold expansion in
Matrigel. In addition, >90% of the UABCs cultured in laminin and fibrinogen gels maintained the expression of cytokeratin 5 after 4 days in culture. We then evaluated the ability of these materials to facilitate the transplantation of UABCs from mice that endogenously express firefly luciferase into immunocompromised NOD scid gamma (NSG™) mice. In preliminary studies, the upper airways of NSG mice were injured using 2% polidocanol and transplanted with UABCs using human fibrinogen and recombinant laminin. These mice exhibited stable bioluminescence for >60 days. We then attempted to transplant human UABCs into NSG mice using fibrinogen gels. Human UABCs were genome edited using CRISPR/Cas9 and AAV to express Nanoluc™ and GFP. UABCs expressing GFP were enriched using flow cytometry to >80% purity and transplanted into mice. Preliminary experiments show that the transplanted human UABCs produce a stable bioluminescent signal for ~120 days. Studies to evaluate the transplantation of UABCs from CF patients that have been genome edited to restore CFTR expression are ongoing.

Selective Targeting Distal Small Airways by AAV Vectors: Implications for Gene Therapy and CF Lung Disease Pathogenesis
Xiaopeng Li, PhD
Michigan State University, Grand Rapids, MI

Cystic fibrosis (CF) is a common genetic disease caused by defects in the cystic fibrosis transmembrane conductance regulator (CFTR), a membrane bound channel permeable to both chloride and bicarbonate anions. Morbidity and mortality rates in CF are largely due to lung disease stemming from chronic bacterial colonization and infection in the conducting airways. Delivering a functional CFTR gene to CF small airway cells holds great promise as a treatment for CF lung disease, as pathological and clinical data suggest that the disease is initiated in small airways with a diameter less than 2mm. Adeno-associated virus 4 (AAV4) is a natural AAV serotype and a safe vector with lower immunogenicity than other gene therapy vectors such as adenovirus. Previously in a CF pig model, we demonstrated that AAV4 has greater tropism for pig small airway epithelia compared with large airways. In addition, AAV4 was superior to all other natural AAV serotypes in transducing ITGα6β4+ pig distal lung progenitor cells. However, the cellular tropism of adeno-associated viral vector 4 (AAV4) in human large and small airways is not clear. We used single cell RNAseq analysis to investigate which cell types AAV4 can transduce in human large and small airways cells cultured at air-liquid interface. We found that AAV4 can transduce similar cell types in both large and small airways, including ciliated, goblet cells. In addition, AAV4 can transduce SCGB3A2+ cells in small airways. These findings support that AAV4 can serve as a suitable viral vector for CF gene therapy to target small airway secretory cells that express majority of CFTR in human small airways.

Novel Models of CF Mucus Plugs for Testing Phage and Tailocin Therapy
Gregory Burkeen, PhD Candidate
San Diego State University, San Diego, CA

The cystic fibrosis (CF) research community is adapting to the wide-scale adoption and remarkable success of CF transmembrane conductance regulator (CFTR) modulator therapy. However, there is still a small patient population of non-responders with ineligible mutations that still requires frequent medical intervention. Due to this development, there is a pressing need to shift towards personalized medicine for patients who are ineligible for CFTR modulators. Researchers need better models of CF lung disease, both theoretical and experimental to make personalized medicine possible. This is necessary to facilitate hypothesis generation as well as testing novel treatment options for possible safety and efficacy issues. I propose that a transition from the current taxonomy-based theoretical model of CF lung microbial ecology to a functional guild-based model may help researchers better understand the fundamental principles of CF lung disease and thus generate novel treatment options.

The new guild-based model consists of four broad functional categories, each of which incorporates many taxa into groups based on their ecological niche. The four basic groups are, Brewers (digest/ferment...
the mucin), Drunks (‘drink’ the fermented mucin and consume lots of oxygen), Putrifiers (produce toxic molecules like putrescine in the anaerobic spaces below the Drunks) and the Nihilists (lone wolf pathogens that kill cells or produce toxins). Using this model allows researchers to generate hypothetical treatment options and predict the changes to the microbial ecology of each patient. There is also an urgent need for novel experimental models of CF lung disease to test these treatment options and verify the expected outcomes on the microbes.

Personalized treatments (such as phage therapy) require personalized models before proceeding to treatment. Currently, the petri dish is still the most common model for testing treatments such as antibiotics or phages for antimicrobial efficacy. I propose an in vitro model based on artificial sputum with alginate added to solidify the microbes and nutrients into a 3D model that replicates a mucus plug in the lower airways of the CF lung. This model is tailored to the individual, by using sputum metagenomes to assist in the isolation of the key members of each guild per patient, then reassembling the isolated microbes into a patient specific model microbial community inside the artificial mucus plug. These mucus plugs are then moved in and out of treatment conditions to simulate a given therapeutic regimen.

**Pseudomonas aeruginosa** Diversifies Inside Cells to Form an Adaptive Niche that Resists Antibiotic Treatment

Naren Gajenthra Kumar, BTech, PhD
University of California, Berkeley, CA

Chronic infection of the airways with *Pseudomonas aeruginosa* (PA) reduces life expectancy and significantly impacts quality of life in individuals with cystic fibrosis (CF). While it is well established that strains encoding the type three secretion system (T3SS) toxin, Exotoxin S (ExoS), invade and replicate in diverse host cell types, the contribution of intracellular bacteria to disease pathogenesis remains unclear. Recently, we found that intracellular PA diversifies within host cells to form sub-populations with distinct profiles of gene expression. Here, bronchial and CFTR deficient cells were infected with WT or T3SS (DexsA) mutants and intracellular populations studied from 4 - 20 hours post-infection (HPI) after killing extracellular bacteria at 3 HPI with amikacin. Using gene expression reporters for the T3SS (exoS-GFP) [acute phenotype], biofilm formation (cdrA-GFP) [chronic phenotype], and inducible GFP [all bacteria], we show that biofilm-expressing PA (cdrA+) coexist with toxin expressing (T3SSon) sub-populations in the same host cell. Vacular bacteria that do not express the T3SS were > 4-fold more resistant (4 μg/mL; 16X MIC) to the cell-permeable antibiotic ofloxacin relative to cytosolic bacteria (0.5 μg/mL; 2X MIC). Intracellular ofloxacin-resistant PA colocalized with lysosomal marker Lamp3 suggesting a role for vacuolar acidification in the development of antibiotic-resistant populations. Ofloxacin resistance of intracellular bacteria in normal and CF cells was independent of biofilm gene expression (cdrA, psl, pel, alginate). Therefore, ‘omics’-based approaches are being used to identify the underlying mechanisms.

In vitro proteomic analysis of ofloxacin resistant intracellular bacteria identified 178 and 126 differentially-expressed proteins in normal and CF epithelial cells respectively. Notable proteins associated with ofloxacin-resistant bacteria included porins (OprE, OprF, OprD) and outer membrane assembly (WbpA, WbpE, Wbpl). Transcriptomic analysis of intracellular ofloxacin-resistant PA showed downregulation of several T3SS genes (popN, pcrH, pcrG, pscB; > 2-fold p < 0.01) correlating with upregulation of a T3SS repressor ptrB (> 2-fold p < 0.001). Significant upregulation of polyamine transport genes (potA–D) (> 2-fold p < 0.05) and putative multi-drug efflux pumps were also observed. Reducing vacuolar acidification using bafilomycin A1 or ammonium chloride reduced the recovery of ofloxacin-resistant populations by ~75% in both normal and CF cells.

Together these findings suggest that *P. aeruginosa* undergoes adaptive phenotypic changes in response to the intracellular microenvironment that favor the development of antibiotic-resistant sub-populations. The extent to which these sub-populations persist in the airways, and if they disperse to infect neighboring tissue in the absence of antibiotics, remains to be determined.
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:

— Parents / Caregivers of Children / Teens with CF – Deborah Menet, LCSW
— Parents / Spouses / Partners / Siblings of Adults with CF – Ann Steiner, PhD
— Adults with CF – Yelizaveta Sher, MD and Kate Yablonsky, LCSW

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

• CFRI Support and Discussion Groups are designed to bring people together to facilitate support, camaraderie and information sharing. 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.

• We understand people have different levels of comfort in participating. For the privacy and security of all, participants are required to turn on their camera and be responsive via microphone or chat.
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.
Glossary

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

**hypoglycemia** — literally meaning “low blood sugar,” hypoglycemia is a condition in which blood glucose levels are abnormally low.
IgE or IgG — a type of antibody level found in the blood that indicates exposure to certain allergens or an immune response.

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.

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

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

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.
Glossary

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

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

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

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

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

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

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

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

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

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

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

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

**sputum** — mucus from the lungs; phlegm.

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

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

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

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

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

Partners in Living ~ Research for Life

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

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

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

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

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

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

- CFRI’s Virtual Gala, “A Breath of Fresh Air,” will be held Saturday, October 15, 2022. Sponsorships are available!

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

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

DANCE LIKE A FOOL 24-HOUR VIRTUAL DANCE PARTY — The inaugural event was held April 1, 2022, with dozens of dancers from across the country logging in and dancing over a period of 24 hours. Seek pledges, dance and support CFRI’s wellness programs.

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

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

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

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

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

Monthly Online Support Groups for the CF Community

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

For Adults with CF
Third Monday of every month, 6:00 pm PT to 7:30 pm. Online Support Group for Adults with CF, which is open to participants nationwide and facilitated by a social worker well versed in issues facing adults with CF.

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

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

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

For Adults with CF Post-Transplant
Fourth Wednesday of every month, 5:00 pm to 6:30 pm PT. This group addresses the unique needs of those with CF who have received a double lung transplant and is open to post-transplant CF adults only. Facilitated by Sonya Haggett, LCSW, adult with CF and lung transplant recipient.
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.

CFRI’s CFQoL Programs are generously supported by Gilead Sciences, Vertex Pharmaceuticals, Genentech, Chiesi USA, Viatris, 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.

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

Generously sponsored by Vertex Pharmaceuticals, Gilead Sciences, Genentech, Chiesi USA, and Viatris.
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 Vertex Pharmaceuticals and contributions to CFRI’s Dance Like A Fool event.

CF Spring and Summer Retreats

The annual CF Spring Retreat and CF Summer Retreat enhance education, positive coping skills, and social support for people who share common experiences with CF, and include educational presentations, exercise, arts and crafts, support groups, and much more. The 2022 summer retreat will be held virtually August 11 – 14.

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. A virtual retreat was held in May, and an in-person retreat in Menlo Park, CA is planned for September 23-25, 2022.

Generously sponsored by AbbVie, Vertex Pharmaceuticals and Gilead Sciences.

CFI Spring and Summer Retreats

The annual CF Spring Retreat and CF Summer Retreat enhance education, positive coping skills, and social support for people who share common experiences with CF, and include educational presentations, exercise, arts and crafts, support groups, and much more. The 2022 summer retreat will be held virtually August 11 – 14.

Join us!

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

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

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

Sponsored by Vertex Pharmaceuticals and Chiesi USA

A Breath of Fresh Air
Virtual Gala Event
On Saturday, October 15, 2022, 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 2022 CFRI Champion.

Sponsored to date by Vertex Pharmaceuticals, AbbVie, Genentech, Chiesi USA, and Viatris

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.