CFRI community
47 Years of Research, Education, Advocacy, & Support
Fall 2022

CFRI's 35th National Cystic Fibrosis Education Conference: Speaker Abstracts

At CFRI's 35th National CF Education Conference, Focus on the Future, held virtually July 29 - 31, 2022, nationally-renowned speakers from across the country shared their expertise and experience on a wide range of CF-related topics. These presentations are now available for viewing on CFRI's YouTube channel. The abstracts below appear in a shortened versio. To watch the presentations, go to https://cfri.tiny.us/CFRI-Conference2022-Playlist

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 a growing health concern among people living with 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 and her colleagues revived a hundred-year-old forgotten cure – phage therapy - to save 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, including the first use of a genetically modified phage cocktail to treat a systemic *Mycobacterium abscessus* infection in a CF patient. In 2018, UCSD launched 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 alternatives 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 prospects for phage therapy for CF patients.

Continued on page 4

Nanotechnologies to Enable Cystic Fibrosis Gene Therapies

Steven Jonas, MD, PhD

Nanoscience and nanotechnology are research areas dedicated to the study of the smallest of stuff. Insight from working at these size scales has inspired the design of the tiniest tools for studying and probing the inner workings of cells, informing new patient-specific diagnostic and treatment approaches. In parallel, the emergence of robust and reliable genome-editing tools, such as the CRISPR/Cas9 system, are 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 genetic cause of the disease is simple: it results from a single gene defect stemming from mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. To establish gene therapy

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CFRI Community
Fall 2022

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Letter from the Executive Director

Dear Friends,

I hope this finds you well during what remains a challenging time for many. CFRI is celebrating 47 years of research funding and service to our community. With your support, we have grown tremendously since 1975, when a group of parents came together to form our organization.

As we go to press, CFRI's Research Advisory Committee is evaluating Letters of Intent for our next round of research awards. Since our first research grant to Dr. Paul Quinton, whose work transformed our understanding of the disease, we have provided millions of dollars in CF research awards. The funding provided by CFRI is often the spark that lights a larger funding flame. In this issue you will see what incredible work is taking place in labs across the country.

CF remains a harsh and capricious disease. In addition to new therapies, members of our community seek diverse resources and services. CFRI provides responsive educational, psychosocial support and advocacy programs to meet ongoing and emerging needs.

As we approach the end of 2022, let us reaffirm our collective commitment to the work that remains to be done. Thank you for your consistent generosity and support.

Warm regards,

Siri Vaeth, MSW | CFRI *Executive Director and Mother of an Adult Daughter with CF*

News from the Board

Dear CFRI Community,

I hope that you are safe and well through the prolonged impacts of COVID-19. As President of CFRI's Board of Directors, I have been inspired by our CF community's engagement and connection throughout the pandemic. I am proud of CFRI's ongoing ability to adapt our programs to ensure access and inclusivity. The Board of Directors and staff have been successful in generating revenue so as as to maintain our quality services. As you will see in the enclosed Annual Report, our recent audit once again confirms that we are a financially stable organization with excellent scale management.

This year we were able to expand our research awards program. This newsletter contains an abstract that describes the innovative work that Dr. Steven Jonas is pursuing at UCLA with the support of CFRI. As a member of our community, you play a key role in bringing this innovative research to life.

Thank you for your ongoing support. With your help we continue to move closer to a cure.

Peace and good health,

Bill Hult | *President, CFRI Board of Directors*

Faces of CF: Serving our Diverse Community

Siri Vaeth, MSW

Imagine going to an emergency room for an urgent and stressful health issue. During intake, when you share that you have cystic fibrosis (CF), the nurse refuses to believe you, insisting this is impossible because you are Asian. Imagine suffering every symptom of CF for decades – from GI to respiratory complications – but your physicians do not test you for CF. The potential diagnosis of cystic fibrosis never occurs to them because you are Black. Unfortunately, these types of experiences are all too common in our CF community. CFRI wants to change this.

> ***"CFRI is committed to inclusion, justice, and equity for all members of the CF community."***

CFRI is committed to inclusion, justice, and equity for all members of the CF community. Our Faces of CF Diversity & Inclusion Program raises awareness among medical care providers and communities of color that CF impacts people of every race and ethnicity, while improving CFRI's resources and support to all those impacted by the disease. Our work is guided by an engaged Cystic Fibrosis Diversity and Inclusion Advisory Committee. Members of the committee represent all groups impacted by CF, and their input is vital in the creation of enhanced outreach, resources, and support.

CFRI recently distributed a survey to cystic fibrosis social workers, asking them to identify what gaps in resources they have witnessed among their patients from underrepresented communities, and in particular, those who do not speak English. From a long list of potential resources (print, online, in-person events, peer-to-peer mentoring, etc.) the most commonly cited gap was the lack of printed resources, particularly in Spanish. CFRI seeks to fill this gap.

Language barriers create challenges for many, and limit access to resources. CFRI has robust YouTube playlists in Hindi and Spanish. We have expanded our print resources, while ensuring our website is fully accessible in multiple languages. We also offer an online monthly support group for Spanish-speaking members of our community, facilitated by a licensed clinical social worker. We have released updated version of our very popular *Fibrosis Quística en la Clase*, and are developing tools and resources related to self-advocacy/advocacy for one's child; mental health; adjusting to a CF diagnosis; and financial assistance programs.

Efforts are focused on improving access and health outcomes. CFRI is reaching out to community health clinics serving diverse communities – targeting those serving the Hispanic/Latinx community –- to distribute information and resource materials to their patients and families to increase awareness of cystic fibrosis in this often overlooked community.

Many members of our community feel isolated. The sense of being rare within a rare disease can be alienating, limit access to resources, exacerbate health disparities, and worsen mental health. CFRI is committed to being a partner to our community in addressing this unmet need.

Faces of CF Diversity Programs is funded through grants from Global Genes, Vertex, Gilead, Viatris, Genentech, and Chiesi USA

Symptoms of Colon Cancer Mimic CF-Related GI Issues

Do not ignore GI issues: Those with CF have far higher rates of colon cancer. If you have these symptoms, talk with your care team about whether you need a colonoscopy.

- *A change in bowel habits (diarrhea, constipation) lasting for more than a few days;*
- *Cramping or abdominal (belly) pain;*
- *Bloating or full feeling;*
- *The feeling that you need to have a bowel movement even after having one;*
- *Rectal bleeding with bright red blood;*
- *Blood in the stool;*
- *Weakness and fatigue*

CFRI's 35th National Cystic Fibrosis Education Conference *Continued from Cover*

Steffanie Strathdee

Dao Nguyen

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

Christine Hachem

With improvements in therapies for CF, there has been an increase in GI cancers as CF is a multisystem disease that involves the GI tract. 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. GI cancers in cystic fibrosis appear at younger ages. Diagnosis is often 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). 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 currently the only recommended screening test for CRC in CF. However, a multicenter trial is evaluating stool-based screening for CRC. 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 are discussed, and screening recommendations for GI cancers in CF are reviewed.

Advances in Gene Therapy for Cystic Fibrosis

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

Paul B. McCray, Jr.

This presentation provides an overview the history of CF gene therapy, from the discovery of the CFTR gene to newer gene delivery strategies and novel gene repair technologies. Advances in the treatment of other monogenetic diseases are also informing CF therapeutic strategies. Despite initial enthusiasm in the 1990s, there are currently no FDA-approved gene therapies for CF. 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. 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. 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

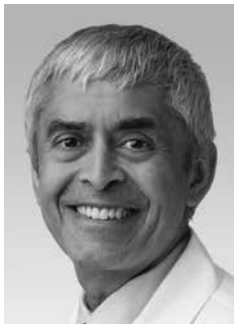
Richard H. Simon, MD
University of Michigan, Ann Arbor, MI

The survival of people with CF is rapidly increasing due to the development of highly effective CFTR modulators. The health challenges of aging with CF obviously overlap with those 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. Another category of problems related to aging are the consequences of past or current CF treatments, for example side effects of antibiotics, particularly intravenous tobramycin. Repeated courses over many years can lead to progressive hearing loss, balance problems, and/or decreasing kidney function. Improved CFTR function due to modulators may also increase the prevalence of some conditions commonly seen in aging populations, such as high blood pressure, high cholesterol levels, coronary artery disease, stroke, and obesity. By implementing the appropriate screening programs and incorporating preventive and treatment measures that work in the general population, the full benefits of the CFTR modulars should be realizable for all people with CF.

Richard H. Simon

mRNA therapy for People with Cystic Fibrosis: Next Steps

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



Manu Jain

Over the last ten years, highly effective CFTR modulators have transformed the lives of many people living with CF because of their unprecedented clinical impact. However, there are still a substantial percentage of people with CF (PwCF) who are not eligible for modulator treatment. 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

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Stephen Aller, PhD

Steven Jonas, MD, PhD

Carlos Milla, MD

Kenichi Okuda, MD, PhD

Matthew Porteus, MD, PhD

Zachary Sellers, MD, PhD

Paul Bollyky, MD, PhD

Nina Pennetzdorfer, PhD

Suzanne Fleiszig, OD, PhD

Naren Kumar, PhD

CFRI Funds Cutting-Edge Research to Move Us Closer to a Cure

With the support of our community, we are providing grants to the following eight researchers. Much of this research will benefit all those with cystic fibrosis, regardless of their CFTR mutation.

New Horizons Award Program:

- **Stephen Aller, PhD**
  University of Alabama at Birmingham
  Role of CFTR Arginine-933 in Folding, Gating and Potentiator Drug Binding
- **Steven Jonas, MD, PhD**
  University of California Los Angeles
  Developing Nanotechnology-Enabled Gene Therapy Solutions to Correct CFTR Mutations in Airway Stem Cells: Toward a One-Time Cure for Cystic Fibrosis
- **Carlos Milla, MD, *Principal Investigator***
  Stanford University
  Improving CF Airway Mucociliary Clearance: Toward Transition from Animals to Humans
- **Kenichi Okuda, MD, PhD, *Principal Investigator***
  University of North Carolina at Chapel Hill
  Pathways Maintaining Basal Mucin and CFTR-mediated Fluid Secretion in the Human Distal Airway
- **Matthew Porteus, MD, PhD, *Principal Investigator***
  Stanford University
  Identifying Biomaterials that Enable the Transplantation of Gene Corrected Airway Stem Cells to Treat Cystic Fibrosis
- **Zachary Sellers, MD, PhD, *Principal Investigator***
  Stanford University
  Targeting IRBIT to Correct Bicarbonate Secretory Defects in Cystic Fibrosis

Elizabeth Nash Memorial Fellowship Program:

- **Paul Bollyky, MD, PhD, *Principal Investigator***
  Nina Pennetzdorfer, PhD, *Postdoctoral Fellow*
  Stanford University
  Targeting Bacterial Resistance to Phage Therapy in Cystic Fibrosis
- **Suzanne Fleiszig, OD, PhD, *Principal Investigator***
  Naren Kumar, PhD, *Postdoctoral Fellow*
  University of California Berkeley
  Characterizing the Intracellular Diversification of Pseudomonas aeruginosa in Chronic Lung Infections

CFRI Is Your Partner in Living

- **MONTHLY GIVING:** *Champions of Hope!* Donations to Champions of Hope provide a revenue stream to support research to find a cure for CF and enhance CFRI's programs in CF education, support and advocacy.
- **TRIBUTES:** *"In Honor Of"* and *"In Memory Of"* — Recognize a loved one with your choice of gift. CFRI will promptly send an acknowledgement letter to your designee.
- **HOLD YOUR OWN EVENT:** Cocktails for a cure, yoga, corn hole, Pictionary challenge – no idea is too big or too small. Create an event, and we'll help you make it happen.
- **FACEBOOK:** Many community members create fundraisers for CFRI by donating their birthdays on Facebook. Go to **https://www.facebook.com/cfri.org/**, scroll down to Fundraisers, and click on Create!
- **STOCK DONATIONS TO CFRI:** Donating appreciated stock avoids capital gains taxes incurred had the stock been sold. You're also entitled to an income tax charitable deduction for the stock gift date's fair market value.
- **SPECIAL EVENTS:** Gala, Golf Tournaments, even a possible Pickleball Tournament – join the community fun.
- **PLANNED GIVING:** Benefits can include increased income, substantial tax savings, the opportunity to meet your philanthropic goals, and the satisfaction of making a very significant gift to CRFI during your lifetime.
- **BEQUESTS:** Include CFRI as a beneficiary in your Will or Living Trust. At the time of your passing, your designated amount would come to CFRI - tax-free to your heirs and CFRI.

For more information, please contact Stacie Reveles, CFRI's Programs and Advocacy Associate: 650.665.7586 or sreveles@cfri.org.

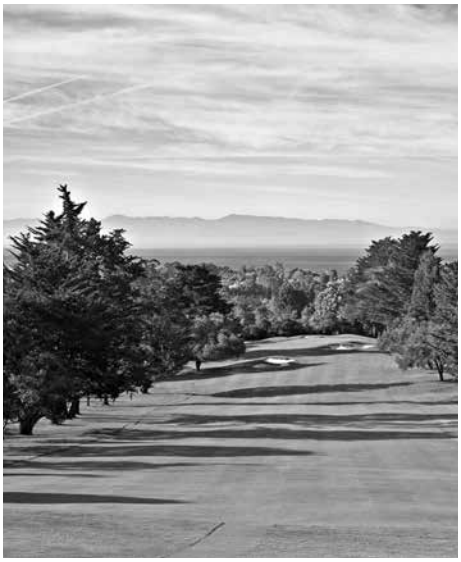
Tributes

Our *"In Memory of"* and *"In Honor of"* pages provide the opportunity to honor a person, or family, or to remember a loved one. If you want your donation to honor or remember someone special, please include the person's name and address with your donation.

At your request, we will send an acknowledgment of your gift to the personyou designate.

Please mail your contributions to: **CFRI**
1731 Embarcadero Road,
Suite 210, Palo Alto, CA 94303

Or go to **www.cfri.org**
to make a donation online.



Community Abounds at the 38th Annual Golf Tournament for CFRI

After 10 years at Pasatiempo Golf Club, this year's Cystic Fibrosis Benefit Golf Tournament returned to its roots – Cinnabar Hills Golf Club in San Jose. On August 15, 160 dedicated golfers gathered to enjoy friendly competition while supporting the search for a cystic fibrosis (CF) cure. Participants had an incredible day, netting over $87,000 for CFRI's research and programs. Of this total, $15,000 is being matched by CFRI's Jessica Fredrick Memorial CF Research Challenge Circle and designated for CFRI's research grant awards.

The event is deeply personal for the event co-chairs, Scott Hoyt and Mike Roanhaus – both have daughters living with cystic fibrosis. CFRI is extremely grateful to Scott, Mike, and the dedicated members of the event committee, Tina Capwell, and Ralph Swanson, and the many participants whose support advances cutting-edge research and much needed support programs for those living with CF. We also thank the long-time major sponsors of the event - Star One Credit Union, the Kirkorian Family Foundation, as well as the Roanhaus family. Dates for 2023 will be announced soon!

In Honor of

April 1, 2022 — October 15, 2022

Sadie Anderson
Bridget and John Barnes
Lucy L. Barnes
Erin Barzanty
Jennifer Belken
Kara Borowski
Ryan Bortz
Isabel Stenzel Byrnes and Andrew Byrnes
CFRI Staff
Crystal Collier
Shaun Collins
Cameron Cornell
Tim Crose
Barbara and Jim Curry
Bradley Daniel
Stacy Dean
Sharon Dunn
Tess A. Dunn
Debbie Duplessis
Janelle and Andre Estournes
Jarrod Fischer
Victoria and Oscar Flamenco
Kathleen P. Flynn
Cassie Frankel and Chris Morrow
Sean Gallagher
Dr. Richard and Charlene Gerlic
Mark E. Gerow
Connor Ghione
Larissa Giuliano
Ellie Goldberg
Elyse Elconin Goldberg
The Goldberg Family
Gianna Gutierrez-Serrato
Sonya Haggett
Alex and Liz Hampton
Melanie Henshaw
Erinn and Kristen Hoyt
The Hoyt Family
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Jimmy and Elizabeth
Darren Johst
Michelle Jones
Harper Jorgenson
Lisa Kennelly
Martin Kharrazi
Franny Kiles
Steven Kusalo
Jane LeBlanc
Alyssa Lenart
Maeve Leonard
Joseph Librers
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Lucy Phillipson
The Powell Family
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The Roanhaus Family
Ann Robinson
Carl Robinson and Family
Cortney Paige Roeder
Taylor Rolefson
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Lauren Russell
Janice Shaul
Robert David Shepherd
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Adam Thompson
Sesh Tirumala
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Dr. Ahmet Uluer
Siri Vaeth
Chris Vallee
Jane Thibault Van Dyke
William Vogt
Devin Wakefield
Scott Wakefield
Natalie Wasserman
Jeff Wine, PhD
Nina Wine
Jonathan Witczak
Laura Witczak
Amanda Wood

In Memory of

April 1, 2022 — October 15, 2022

Marcus, Kimberley and Carol Adelman
All of Stanford's CF Patients we have lost
Gianna Rose Altano
Mary Andrade
Jessica Arvidson
Debbie Ware Babbitt
Patrick Baird
Anne C. Beltrame
Brett Bennett
James W. Bertolini
Wendy Davis Bosarge
Terri Boskovich
Greg Brazil
Alicia Brogle
Kyle Butler
Carol Marden Carey
Karola Carpenter
Cassandra Cochran
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Karen Melvin
Jessica Mobley
Jennifer Montgomery
Ross Moran
Lynn Moyse
Dorothea V. Munro
Mary Murphy-Piro
Elizabeth Nash
Danny and Kevin O'Brien
Michele Denise Olson
Jennifer Ortman
Barbara Palys
Lisa Pearne
Sean Peterson
Catherine Rawlings
Dea Roanhaus
Katie Robinson
Rob Robinson
Pamela K. Rockhold
Tom Rolefson
Randy Rupracht
Joseph Marden Sinnaeve
Tammy Smerber
Anabel Stenzel
Erin Phillips Taylor
Tara Telford
The Thibault Family
June Thompson
Dresden P. Tingley
Lisa Tingley
John Trask
Louis Anthony Trigueiro
Todd Trisch
Freida and Frank Turner
Jennifer Uskoski
Joy Villasenor
Tom Walton
Sean Waltrip
Seymour Weiner
Tara Weir
Maurice Wernli
Hayley C. Wester

Pet Memorials

Avani (dog)
Lady (dog)
Lilo (dog)
Titus (cat)
Toby Trezza (dog)

CF Quality of Life Programs: Supporting the Mental Health of Our Community

Due to its unpredictability, daily treatment burden, and diverse symptoms, cystic fibrosis remains a challenging disease for those diagnosed, as well as for those who love them. Those with CF and their family members have elevated rates of depression and anxiety, and studies show that depression can negatively impact adherence to one's medical regimen. To provide support, CFRI offers a range of programs to address the psychosocial needs of our community.

- **Counseling Support:** CFRI provides up to $120 per session for six sessions of counseling to individuals with CF (children and adults), their parents, partners, spouses, and siblings with the licensed provider of their choice. Participants must live in the U.S.
- **Support Groups — Held Via Zoom, Open to the National and International CF Community, Free, and Facilitated by Licensed Social Workers:**
  - **Caregivers Support Groups:** Two groups are offered – one for parents of children with CF, and another for parents/spouses/partners of adults with CF. The groups are held on the third Tuesday of every month.
  - **CF Adults Support Groups:** Adults with CF are invited to this group, held the third Monday of every month.
  - **Transplant Support Group:** This group is open to CF adults post-transplant. Meetings are held on the fourth Wednesday of every month.
  - **Spanish-Language Support Group:** This group welcomes Spanish-speaking adults with CF as well as family members of adults and children with CF. Facilitated in Spanish, the group meets the second Wednesday of every month.
  - **Teen Support Group:** Teenagers with CF meet the third Wednesday of every month. Parents must give consent for their teens to attend.
  - **CF Bereavement Group:** For those who have lost a loved one to CF, this group includes sharing and discussion, goal setting, grief education, and self-care strategies. The group meets the second Tuesday of each month.
- **Mindfulness and Meditation – Zoom Into Now:** CFRI offers monthly mindfulness meditation sessions for the CF community with Dr. Julie Desch on the fourth Tuesday of every month. Zoom Into Now combines mindfulness practices and meditation, which have been shown to reduce anxiety and depression. Open to those with CF and their family members, 16 years and older.

These programs are offered at no charge to our community members. For more information, visit our website **www.cfri.org**, or email Sabine Brants at **sbrants@cfri.org**.

Partners in Living Initiative — CF Quality of Life Programs are supported through grants from Vertex Pharmaceuticals, Gilead Sciences, Genentech, Chiesi USA, Viatris, individual donors, and contributions through CFRI's CF Quality of Life Program, a Living Legacy of Peter and Kathy Judge.

CFRI's Cystic Fibrosis Wellness Initiative: Virtual Programs to Improve Physical and Mental Health

CFRI's wellness programs were developed in recognition of the positive impact of movement and exercise upon one's physical and mental health. These online programs are free, fun and interactive, and are open to those with CF, as well as their parents, spouses, partners and siblings nationwide. Participants have the opportunity to improve their physical and emotional health while working out in a supportive online environment.

CFRI is offering yoga and physical therapy classes. Our instructors are part of the cystic fibrosis community, and understand the issues faced by those with CF. By exercising together, our community builds emotional and physical resilience while forging new connections and friendships. For the current schedule, go to the events page at cfri.org

CFRI's CF Wellness Initiative is sponsored by Vertex Pharmaceuticals with additional support from the Dance Like A Fool Event donors and sponsors.

Honoring Our Community Heroes

At the 35th National CF Education Conference in July, CFRI proudly honored four remarkable people for their outstanding contributions to the CF community. We are grateful for their time and commitment to those living with cystic fibrosis.

Dave 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. In 2021, Anna was diagnosed with stage-4 colon cancer. She is now 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.

CFRI CF Professional of the Year Award

— Dennis Nielson MD, PhD

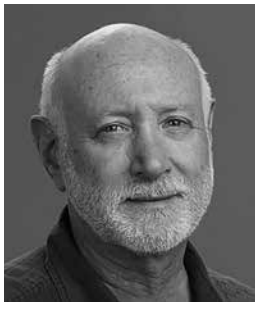
Dr. Nielson is a Professor of Clinical Pediatrics at UC San Francisco, where he served as Director of the Cystic Fibrosis Center at UCSF Benioff Children's Hospital from 2001 until his recent retirement. He completed a pediatric residency and pulmonary training at UCSF Medical Center, and later organized 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.

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. Five years ago she received a life saving double lung transplant. Sonya's clinical practice has focused on community mental health where medical, criminal justice, and aging issues intersect. She has served CFRI over the years as support group facilitator for the CF Adult Retreat and Educational Conference and is a CF 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.

Paul M. Quinton Cystic Fibrosis Research Legacy Award

— Richard Moss, MD



Richard 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. His research interests have included pathogenesis, outcome measures, and treatment of chronic airway diseases of childhood such as asthma and CF, 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 involved with CFRI for decades and has served on CFRI's Board of Directors since 2015. 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.

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SAVE THE DATES!

Please sign up to receive our weekly eNewsletter to to stay informed of our many programs and events!

New Program: Zoom Into Now
November 22 / December 27
January 24 / February 28
March 28
Monthly online Mindfulness and meditation sessions for the CF community with Dr. Julie Desch. Open to those with CF and family members, 16 years and up

CF Virtual Support Groups
See dates on page 8

CF Spring Break
March 2023
A virtual retreat for adults with CF, their friends and family

Purple Hair Challenge
May 2023
Color your hair purple and challenge your friends to raise CF awareness!

Embrace Mothers' Retreat
May 5 – 7, 2023
An in-person retreat for mothers of children and adults with CF
Vallombrosa Retreat Center
Menlo Park, CA

CFRI 36th National CF Education Conference
July 28 – 30, 2023
The Conference will be held as a hybrid event (virtual and in-person) in 2023

CF Summer Retreat for Adults with CF
August 2023
More details coming soon!

For information or to register for these events, please visit our website ***www.cfri.org****, email* ***cfri@cfri.org*** *or call* ***650.665.7559****.*

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Marina Gonzales
Hotel San Luis Obispo
Kayak Connection
Little River Inn
Local Tastes of the City Tours
Lovejoy's Tea Room
McLaren Racing Team: Zak Brown
Paula Meier
Mendocino Botanical Gardens
Mendocino Cafe
Mendocino Hotel
Alexandra Mendoza and Steve Ehrsam
The Mob Museum
Museum of 3D Illusions
Napa Valley Bike Tours
Origin Jewelry
Pajaro Dunes Resort
Picchetti Winery
Pinot's Palette
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San Francisco Cable Cars
San Francisco Giants
San Joaquin Country Club
Santa Cruz Beach Boardwalk
Seaside Company and Chardonnay Sailing Charters
Sheraton Hotel Redding
Simoncini Vineyards
Soquel Vineyards
Swimply
Turtle Bay Exploration Park
Marc Uharriet
Walt Disney Family Museum
Windsor Vineyards
Wynn Resort Las Vegas
Yeti

Jessica Fredrick Memorial CF Research Challenge Circle and Fund

Real generosity toward the future lies in giving all to the present. — Albert Camus

Members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle give generously to inspire others to join the search for new CF therapies and a cure. This year to date, Circle members have contributed over $90,000 so as to match – dollar for dollar – donations from individuals committed to CF research. Together, these donations are used for our CF research awards.

Our Circle is named in memory of Jessica Fredrick, who lost her battle with CF at the age of 21. There is still no cure for CF. We need your help to improve and save the lives of our loved ones. Please join this inspiring group! Become a member of the Jessica Fredrick Memorial CF Research Challenge Circle by making a minimum gift of $2,500. You will inspire others to make the dream of a CF cure a reality.

If you are unable to join the Circle, please consider making a gift to the Research Challenge Fund, which will be designated for CF research awards. By giving all to the present, you are generously supporting the future hopes of those with CF.

Disparities in the Diagnosis and Treatment of Asian Patients with Cystic Fibrosis

Researchers at Stanford University have analyzed registries maintained by Cystic Fibrosis Canada, the UK Cystic Fibrosis Trust and the Cystic Fibrosis Foundation to determine whether cystic fibrosis was more prevalent in South Asian communities than other Asian populations. Of the 3.3% of Asian patients in the UK registry, 88% were South Asian. Of the approximate 1% of Asian patients in the Canadian registry, 68% were South Asian. The researchers (S. Vaidyanathan, A. Trumbull, L. Bar, M. Rao, Y. Yu, and Z. Sellers) then examined the CF causing variants among Asian patients. While approximately 90% of White CF patients have at least one copy of the F508del mutation, only 30% to 40% of Asian patients have this mutation. In fact, the researchers found that up to 47% of South Asians are affected by two variants that are not responsive to CFTR modulators. Because many state newborn screening panels only test for CF mutations most commonly found among people of European descent, non-White patients with CF are missed, thereby delaying diagnosis and access to CF therapies and care.

The authors note that based on the estimated prevalence of CF in South Asians, there may be approximately 26,000 – to 146,000 patients with CF in South Asia. They also observe that it is likely that the true impact of CF in Asians may be underestimated, "owing to the high mortality associated with untreated CF."

To read the article, "CFTR genotype analysis of Asians in international registries highlights disparities in the diagnosis and treatment of Asian patients with cystic fibrosis," go to https://tinyurl.com/2p8ux2ns

Embrace ~ A Retreat for Mothers of Children and Adults with CF

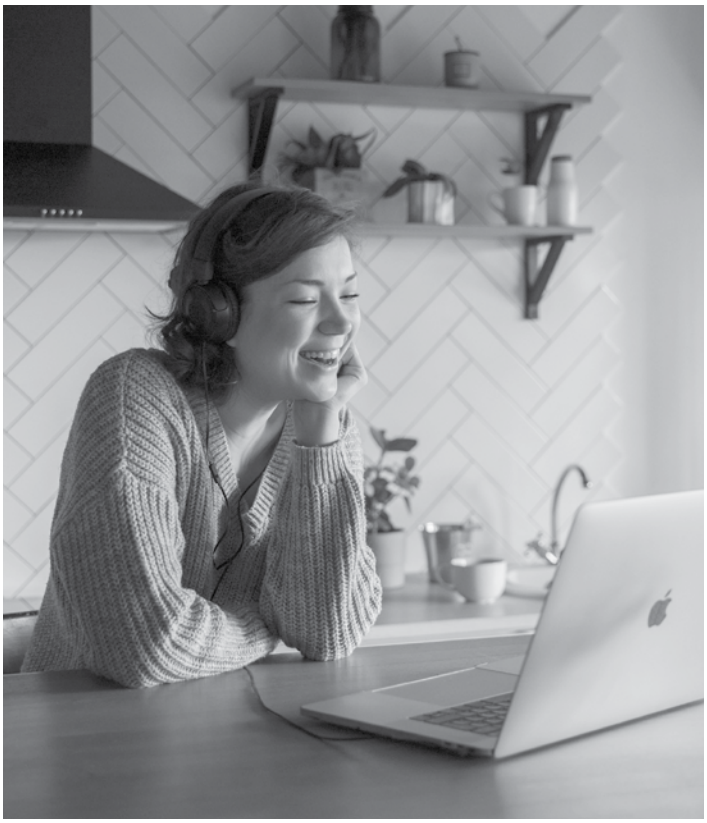
CFRI's 8th annual Embrace Mothers Retreat was offered virtually on May 7, and in-person the weekend of September 23 - 25, at Vallombrosa Retreat Center in Menlo Park, California. This was CFRI's first in-person event since the start of the pandemic. The virtual event was attended by 30 mothers from 17 states, while the event at Vallombrosa was attended by 23 mothers from 8 states. At both events, attendees participated in presentations, therapeutic art and writing workshops, yoga, and an overview of additional resources offered by CFRI to provide lasting support.

Studies show that mothers of children with cystic fibrosis have elevated rates of anxiety and depression, which can directly impact their children's outlook and adherence to their medical regimen. Evaluations of Embrace attendees show that the retreat is extremely effective in lowering symptoms of depression and anxiety. Participating in workshops and activities while connecting with others who share the CF path helps mothers to build resilience for the ongoing challenges presented by this disease.

We are very grateful to our sponsors: Vertex Pharmaceuticals, AbbVie, and Gilead Sciences.

CFRI's Virtual Retreats for Adults with CF Keep Our Community Connected

Nearly 70 adults with cystic fibrosis (CF), along with family and friends, attended the CF Summer Retreat, held August 11-14, 2022. Participants represented 28 states, the United Kingdom and Australia. Retreat provides a welcoming community for adults with CF looking for connection, information, and camaraderie with their peers.



The retreat was fully virtual, and provided a wide array of health-related and psychosocial support programs and activities. In addition to exercise activities tailored to individuals' unique capacities, and a phenomenal art workshop led by artist Dominic Quagliozzi, who lives with CF, participants heard from experts in the field from the U.S., Ireland and Scotland. These educational workshops on dental health, CF- related diabetes, CF-related arthritis, bone health, and physical therapy strategies to address pain were highly interactive. In addition, there were daily rap sessions (support groups) to support positive mental health, and dedicated time to connect, network, and socialize with others.

Attendees reported that the retreat offered new information about CF therapies and treatments, dramatically improved psychosocial health, and provided resources and strategies for coping with the daily challenges of CF. Said one participant: "I don't know how I could get through the year without this amazing program. Being able to connect with the community has helped my mental health greatly. I get so much great information from all the wonderful speakers; it helps to give me added tools to use in care and treatments."

CF Summer Retreat was generously sponsored by Gilead Sciences, Vertex Pharmaceuticals and AbbVie.

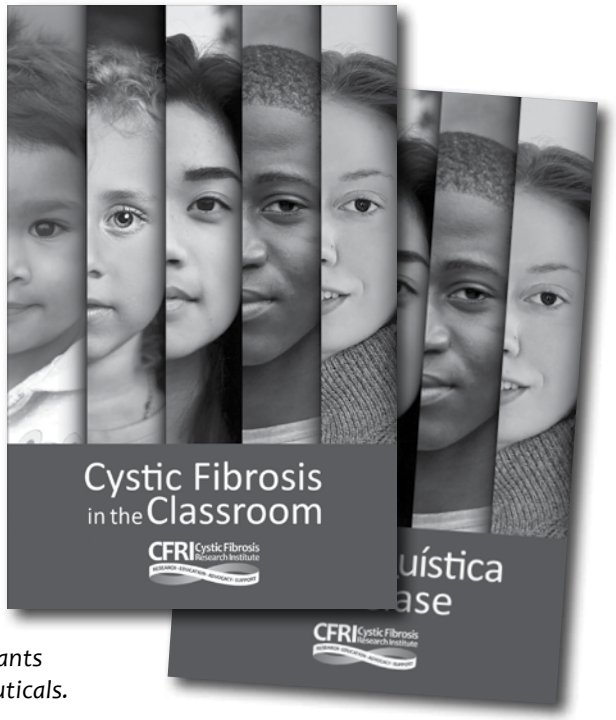
Mark your calendars for the virtual Spring Retreat, which will be held March 17 – March 19, 2023.

CF in the Classroom / Fibrosis Quística en la Clase *Now Available*!

CF in the Classroom, and its Spanish-language equivalent, *Fibrosis Quística en la Clase*, are among CFRI's most popular printed resources. We have recently updated these booklets, which were written to provide vital information to parents of students with CF, students with CF, CF centers, medical clinics, teachers, and other interested parties that engage with students living with cystic fibrosis. The purpose of the booklets is to provide students living with CF and their peer groups, teachers, school administrators, and healthcare professionals, the information necessary to understand potential challenges and help them successfully navigate the school environment from pre-school through college – when living with CF. The booklet contains thorough and relevant information and resources to enable the student living with CF to maintain as healthy a life as possible through adherence to their medically prescribed health-care regimen while engaging in normal school activities.

Physical copies can be requested and CFRI will ship at no cost to individuals as well as in bulk to CF centers. Email cfri@cfri.org to place an order. Digital versions may be downloaded from CFRI's website.

Made possible with educational grants from Viatris and Vertex Pharmaceuticals.



Many Voices ~ Big Impact: CFRI's Advocacy and Awareness Efforts Benefit the CF and Rare Disease Communities *Siri Vaeth, MSW*

Through its Many Voices ~ One Voice Cystic Fibrosis Advocacy and Awareness Program, CFRI engages with our cystic fibrosis (CF) community to raise awareness among the general public and legislative sectors about the burdens and complications of the disease, policies that negatively impact our community, and the need for further support for CF research.

In recent months, CFRI advocates have participated in virtual meetings with elected representatives and their aides at both the state and federal level. At the federal level we encourage increased financial support for the Food and Drug Administration (FDA) and National Institutes of Health (NIH). We are also advocating for passage of the bipartisan PASTEUR Act, to fund research to combat the increasing threat of antimicrobial resistance.

CFRI believes that when members of our community use manufacturer co-pay cards to access their medications, these funds should be applied to their deductibles and out of pocket maximums. On both the state and federal level, CFRI has been involved with coalitions to address the proliferation of co-pay accumulator programs that are now included in many health insurance plans. These do not allow payments from co-pay assistance cards to be applied toward deductibles and out-of-pocket expenses.

As a result, many individuals with CF and their families are shocked to find that they still have a large deductible to meet months into the calendar year. In essence, the deductible is paid twice. Many cannot absorb this cost and are unable to access their needed medication.

Those who rely on specialty medications are most impacted by these policies. It has been found that one in four people with CF have delayed seeking care or skipped treatments because of costs related to insurance premium rates, deductibles, out-of-pocket expenses, and co-payments. CFRI is working with others to support legislation that will mandate that all payments for prescriptions be applied toward one's deductible and annual out-of-pocket total. This type of legislation has already passed in a dozen states and Puerto Rico, with broad bipartisan support.

On the state level, we continue to focus on the creation of Rare Disease Advisory Councils (RDACs), which provide the rare disease community with a formal platform and official voice at the state level to help advise state officials on policies and services that impact us. CFRI is a member of several coalitions working to advance these efforts.

CFRI also continues to support improvements in state newborn screening programs. Cystic fibrosis impacts people of every race and ethnicity, and individuals from underrepresented groups are more likely to have rare mutations that will not be detected through many states' current screening panels.

CFRI invites our community to engage with our advocacy efforts. Please email Stacie Reveles at sreveles@cfri.org for more information.

CFRI's Many Voices ~ One Voice CF Advocacy and Awareness Program is funded through educational grants from Vertex Pharmaceuticals, Genentech, Gilead Sciences, Ionis Pharmaceuticals, and AbbVie.

From Colon Cancer to CF Tattoos, Phage Therapy to Diversity in Our CF Community: CF Community Voices Has Something for Everyone

By the community and for the community, CFRI's video podcast program, CF Community Voices, was created to share information and insights about a wide variety of topics, including CF and inflammation, health insurance options, colon cancer awareness, conference presentations, and more. Some podcast episodes feature inspirational stories, from Athletes of the Transplant Games to Random Acts of Kindness. In addition, there are videos from our Diversity and Inclusion Initiative. Many of the episodes are available with Spanish and Hindi subtitles, as well as captioning in English for the hearing impaired. New episodes are released monthly and can be watched on CFRI's YouTube channel or downloaded from cfri.podbean.com. We look forward to sharing our community's diverse voices. *Generously sponsored by Vertex Pharmaceuticals, Chiesi USA, Gilead Sciences, Genentech, and Viatris.*

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. Dr. Jain provides 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


Donald L. Chi

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 advances in therapies for cystic fibrosis have accelerated considerably in the last decade, there remain gaps in CF treatment. Some people with CF may use complementary and alternative therapy or medicines (CAM) to help address certain symptoms such as insomnia, constipation, anxiety, or overall health. Some commonly reported used CAM among people with CF include probiotics, melatonin, omega-3 fatty acids, curcumin, as well as combination products that claim to improve lung health. CAM are processed by the body in similar pathways as conventional medicines and may pose risk of drug interaction(s) or effect organ systems such as the liver over time. Historically, use of CAM has been viewed by clinicians in a fashion that discouraged open communication between care teams and people with CF 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. It is important to evaluate and discuss available data, including how they apply to a given individual with regards to risk versus benefit, and if elected, determine measures to appropriately monitor therapy. This session reviews 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.


Hanna Phan

CF-Related Bone Disease: Current Evidence and Future Directions

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


Melissa S. Putman

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. 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, weight bearing activity, 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.

Advocacy in the CF Realm

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


Jacob Fraker


Diane Shader Smith

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 California Capitol, provides 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 addresses some of the key issues that are currently impacting the CF and rare disease community across the United States.

Part two features 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, but she has recently

layered on the larger issue of antimicrobial resistance, 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. She talks about the need for new antibiotics, antifungals, and phage therapy, the Pasteur Act, and the groups and efforts that have mobilized to respond to this health crisis.

Advances in Lung Transplantation

Joseph Pilewski, MD
University of Pittsburgh, Pittsburgh, PA


Joseph Pilewski

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. 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.

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

Emily Kramer-Golinkoff is an adult with CF 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 CF. 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. 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, she co-founded Emily's Entourage in 2011 with her friends and family, with the goal of accelerating research and drug development for those in the final 10%. 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.

Nanotechnologies to Enable Cystic Fibrosis Gene Therapies *Continued from Cover*

solutions for diseases like CF, our multidisciplinary team develops and applies nanotechnologies designed to assist with the delivery of gene editing bio-molecules directly into cells to correct the defective gene.

To do this successfully for CF, we need to target self-renewing airway basal stem cell (ABSC) populations for gene correction, which is especially difficult because of their protected location within airway tissues. Our approach to overcome this obstacle involves the design and testing of nanoparticles engineered to open access to the ABSCs and the development of the CRISPR/Cas9-based gene-editing cargoes for CFTR correction. The nanoparticles serve as a nanoscale "shipping containers" for packaging the CRISPR/Cas9 reagents and delivering them to the exposed ABSCs (Fig. 1).

We are developing strategies to deliver the CFTR-correcting nanoparticles as aerosols to enable inhalable gene therapy solutions for CF. To test this capability, we use vibrating mesh nebulizer devices to generate nanoparticleladen aerosol droplets that are directed to ABSCs supported on air liquid interface culture models that mimic the epithelial structure and organization of the human airway.

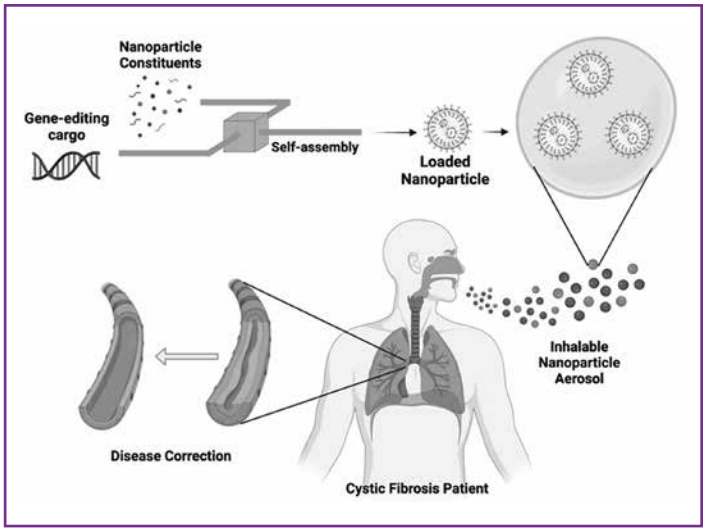


Fig. 1 Schematic outlining strategies for nubulized delivery of naoparticles engineered to enable inhalable gene therapy strategies for cystic fibrosis.

We are evaluating the degree of ABSC exposure and the requirements for achieving functional CFTR-gene correction using these delivery tools. The suite of nanotechnology-enabled tools and methods developed here serve as building blocks for the CF research community to bring innovative gene therapy solutions to our patients, accelerating progress toward our collective goal to find a permanent cure for CF on the steepest gradient possible.

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CFRI Mission

To be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support.

CFRI Vision

To find a cure for cystic fibrosis while enhancing quality of life for the CF community.

For their generous support of ***CFRI Community,*** special thanks to: **Vertex Pharmaceuticals, Genentech, Gilead Sciences, AbbVie, Chiesi USA, Viatris,** *and* **Translate Bio**

Visit our website at:
www.cfri.org
for more information about us and about cystic fibrosis.
Call toll free: 855.cfri.now

CFRI's *A Breath of Fresh Air* Virtual Gala A Success!

CFRI's annual gala, held virtually on October 15, 20212, brought together a community united in the search for a cystic fibrosis cure. A lively pre-gala event was attended by community members across the country providing the opportunity to meet with old friends and new. The official gala program launched with a warm welcome from inspiring Canadian triathlete Lisa Bentley, while our emcee, Emmy-award winning Chris Chmura of NBC Bay Area, guided us through a fast-paced but moving program which shared the experiences of dozens of CF community members.

We honored Ahmet Uluer, DO, MPH, Director of the Adult Cystic Fibrosis Program at the combined Boston Children's Hospital and Brigham & Women's Hospital Adult Cystic Fibrosis Program, as our 2022 Cystic Fibrosis Champion. Warm thanks to all who participated in the production of our gala – individuals with CF, parents of those with CF, care providers, and researchers. The auction led to spirited bidding, while donations to our Bid for a Cure grew. By the end of the evening, over $120,000 was raised to support CFRI's research, education and support programs, and $40,000 of this total is being matched dollar-for-dollar by members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle and designated for our CF research awards.

We are grateful for our generous sponsors, in-kind donors, attendees and hardworking Gala Committee members. Everyone played a role in our virtual gala's amazing success — it was truly *A Breath of Fresh Air!*

Please see page 10 *for a complete list of our sponsors and in-kind and auction donors.*

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