CFRI community

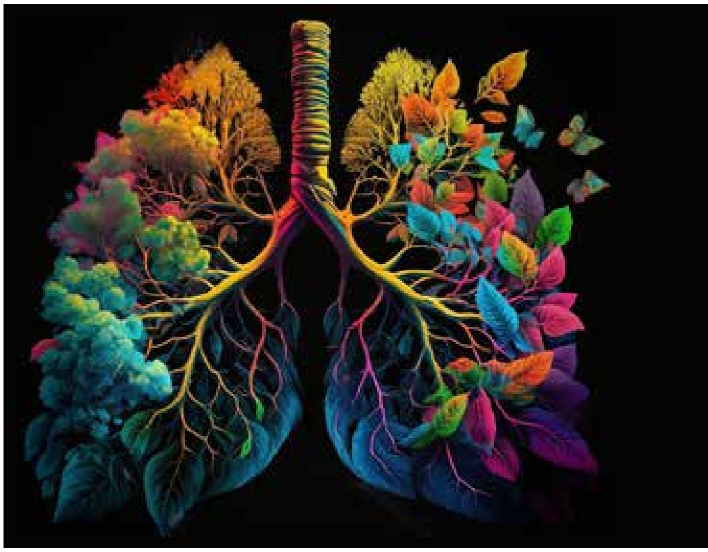
Celebrating 50 Years
CFRI Cystic Fibrosis Research Institute
RESEARCH · EDUCATION · ADVOCACY · SUPPORT
1975 - 2025

50 Years of Research, Education, Advocacy, & Support
Spring 2025

Aging in the New Era of Cystic Fibrosis: Patrick Nash Fellows Training Program Seeks New Models of Multidisciplinary Care

When you read the words "person with cystic fibrosis," what image comes to mind? Quite likely you pictured a younger person, because until recently, those with cystic fibrosis (CF) often lost their lives at a tragically young age. But therapeutic advances – most notably CFTR modulators – have extended lives for many. While cause for celebration, this longevity is often fraught with new health challenges. Some are related to aging alone, but many – gastrointestinal complications, diabetes, osteoporosis, cardiovascular disease, and cancer – are caused or exacerbated by CF. CFRI seeks to explore emerging needs, expand research, and develop improved clinical care strategies to address the multifaceted impacts of CF and aging. To advance these efforts, the Patrick Nash Fellows Training Program, Aging in the New Era of Cystic Fibrosis, was born.

This new program honors Patrick Nash, a beloved member of CFRI's CF Adult Advisory Committee who provided support to his fellow adults with CF, while contributing ideas and insights to enhance CFRI's program offerings. Patrick was intensely interested in the emerging health issues faced by adults with cystic fibrosis. He and Dr. Ahmet Uluer were exploring strategies to expand research and improve coordinated care when Patrick was diagnosed with aggressive pancreatic cancer. He lost his battle in late 2022. Patrick's death inspired the formation of a dedicated steering committee inspired to develop the Patrick Nash Fellows Training Program in his memory.



In its inaugural year, the program brought together providers from multiple disciplines to increase their understanding of the many non-pulmonary CF-related comorbidities that present or progress in adulthood. Sixteen post-doctoral fellows, representing 15 institutions and 9 medical disciplines, attended the symposium in Denver, Colorado, where a faculty of highly recognized clinician-researchers provided seminars on the multiple facets of care for adults with CF, with time for robust discussion. The lived experience of those with CF was incorporated, through presentations (via Zoom)

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CFRI-Funded Research In Action:

A Hybrid Gene Correction Strategy for Cystic Fibrosis

Dr. Amaya and Dr. Porteus

Anais K. Amaya, PhD, Matthew H. Porteus, MD, PhD / Department of Pediatrics / Stanford University

Recent advancements in small molecule correctors and modulators have revolutionized treatment options for most people with CF. These therapies target the underlying protein dysfunction caused by mutations in the CFTR gene, restoring partial function to the defective channels. Despite these advances, a subset of people with CF remains without effective treatment options due to genetic mutations not responsive to available modulators, side effects, or other complicating factors. Additionally, current modulator therapy requires daily administration to maintain therapeutic benefit.

A proposed therapeutic approach with potential for permanent benefit involves directly repairing the genetic mutation responsible for CF using genome editing technologies such as CRISPR/Cas9. This strategy aims to modify the person's DNA to restore normal CFTR gene function, potentially offering a one-time intervention with durable therapeutic effects. By addressing the root genetic cause rather than managing downstream protein dysfunction, CRISPR-based therapies could overcome the limitations of current treatments and provide options for people with CF currently underserved by available medications.

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CFRI Community
Spring 2025

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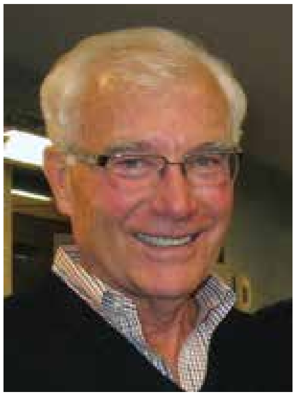
Greetings from the President of the Board and the Executive Director




Dear CFRI Community,

We hope that you are safe and well. CFRI is an organization by and for the cystic fibrosis community. Recent events have created a great deal of concern regarding funding for scientific research, and specifically CF-related research. Our mission-driven Board, staff and volunteers remain focused on addressing the diverse needs of our community. As such, we will expand our research funding awards in 2025, to provide support to 10 innovative projects at universities and medical institutions across the country. We also remain steadfast in our work to raise awareness of the challenges of cystic fibrosis, ongoing disparities in diagnosis and care, and the importance of working collaboratively to improve longevity and quality of life.

Our newest program, the Patrick Nash Fellows Training Program represents another jewel in our community-focused crown. Through this program we are creating a national network of multidisciplinary medical clinicians and researchers to improve care – and care models - for adults with CF as they age. CF remains a harsh and capricious disease. Approximately 10% of our community members cannot use CFTR modulators, and people of color are overrepresented in this group. Lung transplant recipients need better therapies. There is a great deal of work to be done and CFRI seeks innovative strategies to address emerging needs.

Each one of us plays a part in the search for a cure and we thank you for being a part of this engaged and caring community. Together we accomplish great things.

Bill

Bill Hult | *President, CFRI Board of Directors*
Siri Vaeth, MSW | *Executive Director*

Happy Birthday CFRI!

Celebrating 50 Years of Research and Service!

This year, CFRI celebrates five decades of cystic fibrosis research progress and community engagement. CFRI was founded in 1975 by a small group of parents whose children were not expected to survive to adulthood. Over the past 50 years, tremendous advances in CF therapeutics have improved and extended the lives of those with CF. With the support of our community, CFRI has played a pivotal role in these advances. But we are not done yet.

CFRI is proud to fund innovative research while providing educational and psychosocial support to those impacted by this still challenging disease. CFRI is by and for the CF community!

To celebrate CFRI's important milestone, we will host a special 50th anniversary Gala in October – for details, see page 4. ***Join us, and stay tuned for other activities to celebrate our golden anniversary!***

The Many Faces of CF

Nearly 90 years ago, in 1938, Dr. Dorothy H. Anderson published "Cystic Fibrosis of the Pancreas and Its Relation to Celiac Disease." This groundbreaking work was the first published description of cystic fibrosis and notably highlighted the disease's racial and geographic diversity. Over time, however, CF became associated primarily as a condition impacting Caucasians. While recent efforts have broadened understanding—recognizing that CF affects individuals across all races and ethnicities—this outdated association has contributed to delays in diagnosis, underrepresentation in clinical trials, limited treatment advancements, and medical stigma.

CFRI's Faces of CF Diversity and Inclusion Program addresses these gaps in CF care by creating resources that educate, empower and advocate for individuals with CF and their families across diverse communities. This initiative is guided by CFRI's active Diversity and Inclusion Advisory Commitee, which includes people with CF, family members, caregivers, and clinicians. Through their lived experiences and professional expertise, CFRI is able to develop and distribute resources in multiple languages, covering a wide range of topics, all created by and for the CF community.

Throughout 2025, CFRI will be hosting quarterly workshops to promote inclusive, collaborative CF care. The workshops, open to patients, families, and CF care team members, will progress from personal mindfulness to inclusive communication, culminating in strategies to expand research participation through diversity and equity practices. Recordings and resources from the workshops will be available to registrants and the public on CFRI's CF Diversity & Inclusion YouTube playlist, ensuring broad access.

Additionally, CFRI continues to publish videos on topics of importance to the CF community with Spanish and Hindi subtitles. We create video and print media that directly addresses CF diagnosis, treatment, and care across the African American, Hispanic/Latinx, East Asian, and South Asian communities. These resources can be found at **www.cfri.org/diversity-and-inclusion**, with printed copies available upon request at no cost.

CFRI offers many support groups to directly address the challenges faced by all people with CF, and specifically, those of diverse racial and ethnic backgrounds—including adults who received a late diagnosis, those who cannot access modulator therapies, or have undergone a transplant. CFRI has recently updated its group for the Spanish-speaking CF community. The monthly 'Conocimiento y Conexión' is a virtual community gathering that spotlights a guest speaker and educational topic, to address the unique issues faced by Hispanic/Latinx individuals and families affected by CF.

As CFRI celebrates 50 years of research, education, advocacy, and support, we proudly look toward a future where diversity, inclusion, justice, and equity for all individuals with CF are fully embraced and realized.

CFRI's Faces of CF Program is made possible through grants from Viatris, AbbVie, ReCode, Gilead Sciences, and Genentech.

CFRI's Online CF Wellness Classes: Get Moving and Improve Your Physical and Mental Health!

CFRI's wellness program was developed in recognition of the positive impact of movement and exercise upon one's physical and mental health. All our wellness classes are free, fun and interactive. Anyone with CF, as well as their parents, spouses, partners and siblings from across the country – and globe - are welcome to join. Participants have the opportunity to improve their health while working out in a supportive online environment with their CF community peers.

Boost your physical and mental health! Classes are held on alternating Thursdays (4:00 pm PT / 7:00 pm ET) and Saturdays (9:00 am PT / 12:00 pm ET), and offer a range of activities, from Slow Flow Yoga and Mat Pilates to U-Jam, strength building, and mobility. You can register for the Thursday and/or Saturday track, and attend as many classes as you would like. You will receive a reminder with a link within 24 hours of the launch of each class.

No experience is required; all abilities and mobilities are welcome! For the complete schedule and to register, go to **cfri.org/wellness-classes/**.

CFRI's CF Wellness Classes are sponsored by Vertex Pharmaceuticals and Viatris, with additional support from individual donors through CFRI's Dance Like a Fool event.

A Breath of Fresh Air ~ Celebrating 50 Years of Service to the CF Community *A Gala to Support CF Research, Education, Support & Advocacy Programs*

Saturday, October 11, 2025

Save the date! CFRI's Breath of Fresh Air Gala will take place on Saturday, October 11, 2025, at the elegant Hillsborough Racquet Club (Hillsborough, California). Join us for this exciting evening event to celebrate CFRI's 50th anniversary, our strong community and exciting research progress! Sip extraordinary wines while enjoying delicious food, music, inspiring stories of hope, and an exciting live auction. Help us to honor the 2025 CF Champion, Dr. John Mark of Stanford, whose patient-centric care has improved the lives of countless individuals with CF. In addition to this in-person soirée, our community members from afar can watch a virtual gala presentation and bid in our online auction that includes a wide array of get-aways, art, wine, and exciting experiences.

Take a deep breath and be inspired to support the search for a CF cure! All proceeds will benefit CFRI's research, education, advocacy, and support programs to improve the lives of those with CF.

Sponsorship opportunities are available. For more information go to — **www.cfri.org**, or call **650.665.7586**.

Our thanks to our Hillsborough Racquet Club hosts, Jim and Barbara Curry. Generously sponsored to date by Vertex Pharmaceuticals, AbbVie, ReCode, Viatris, and our Media Sponsor, NBC Bay Area.

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

CFRI will host its 11th Embrace Mothers Retreat from May 2 – 4, at the beautiful Vallombrosa Retreat Center in Menlo Park, California. Through art, yoga, writing and advocacy workshops, the retreat offers an opportunity for women who share the CF path to connect and rejuvenate.

The theme of this year's Embrace event is "Restoring Our Wholeness." The diagnosis of cystic fibrosis can initially be shattering. Through information, community connection and support, the pieces may be restored, though in an altered form. In recognition of this process, the Retreat includes a special Broken Bowl® workshop, based on the ancient Japanese art of kintsugi. Guided by trained facilitators, participants will break, restore, and decorate a ceramic bowl, mending the cracked seams with gold, creating a powerful symbol of participating mothers' CF journeys.

The Embrace Retreat was created to address the high rates of depression and anxiety that have been documented among mothers of children with CF. Sustained stress is emotionally and physically damaging, and this can directly impact their children's outlook and adherence to their medical regimen. Women travel from across the United States to attend, and through the years a supportive network has been created. Evaluations of Embrace participants show that the retreat is extremely effective in lowering symptoms of depression and anxiety.

For more information, please visit our website at **www.cfri.org**.

Embrace Mothers Retreat is generously sponsored by Vertex Pharmaceuticals and AbbVie.

Learn and Be Inspired: 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 as well as inspirational stories from within the CF community. Recent episodes address issues including CF and cancer, coping with medical trauma, and the experiences of double lung transplant recipients. 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 downloaded on CFRI's podhosting site: **cfri.podbeancom**. You can also watch on CFRI's YouTube channel: **https://curecf.cfri.org/YouTube** We look forward to sharing our community's diverse voices.

CFRI's 2025 CF Community Voices is made possible to date with support from Viatris and Vertex Pharmaceuticals.

Jessica Fredrick Memorial CF Research Challenge Circle and Fund: Advancing CF Research

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. Last year, members of our circle contributed over $100,000 to match – dollar for dollar – donations from individuals designated to CF research. Together, these donations supported our New Horizons and Elizabeth Nash Memorial Fellowship CF research awards.

Please join this inspiring group! Become a member of the 2025 Jessica Fredrick Memorial CF Research Challenge Circle by making a minimum gift of $2,500. You will help inspire others to make the dream of a CF cure a reality. Our Circle was initiated by Suzanne Freiley, whose beloved niece, Jessica Fredrick, lost her battle with CF at the age of 21.

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. In doing so, you help advance the search for a CF cure.

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

At the core of CFRI's mission is to pursue a cure for cystic fibrosis. We invest in the highest quality and most innovative scientific research that will increase understanding of the disease, broaden treatment options, improve quality of life, and expand the search for a cure.

Members of CFRI's Research Advisory Committee (RAC) are currently vetting a diverse range of high-quality research proposals. Their recommendations will guide CFRI's Board of Directors, which votes on 2025 research grant awards in April. Awardees will be shared with our CF community in late May.

Current CFRI-funded researchers include:

New Horizons Award Program

- **Nadia Ameen, MD**
  Yale University School of Medicine
  Elucidating the Ion Transport Functions of CFTR High Expresser Cells (CHEs) and its Relevance to Intestinal Disease in Cystic Fibrosis
- **Benjamin Chan, PhD**
  Yale University School of Medicine
  Optimization of Phage Therapy to Reduce Pseudomonas-Induced Inflammation in Cystic Fibrosis
- **Daria Van Tyne, PhD**
  University of Pittsburgh
  Optimization of Activity and Improved Delivery of Bacteriophages Targeting Burkholderia spp
- **Katrine Whiteson, PhD**
  University of California Irvine
  Targeting Recalcitrant CF Pathogens with Phages, Antibiotics, and Small Molecule Adjuvants
- **Feng Yuan, PhD**
  University of Iowa
  Dissecting Pulmonary Ionocyte Subtypes and their Functional Roles in Cystic Fibrosis

Elizabeth Nash Memorial Fellowship Program

(For Post-Doctoral Fellows)

- **Steven Jonas, MD, PhD, *Principal Investigator***
  Ruby Sims, PhD, *Postdoctoral Fellow*
  University of California Los Angeles
  Designing a Cystic Fibrosis Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cell-Derived Ionocytes
- **Ron Kopito, PhD, *Principal Investigator***
  Celeste Riepe, PhD, *Postdoctoral Fellow*
  Stanford University
  Pharmacogenomic Discovery of Therapeutic Targets for Corrector-Resistant Cystic Fibrosis
- **Matthew Porteus, MD, PhD, *Principal Investigator***
  Anais Amaya Colina, PhD, *Postdoctoral Fellow*
  Stanford University
  A Hybrid Gene Correction Strategy for Cystic Fibrosis

All the researchers listed above will present their work at CFRI's 38th annual National Cystic Fibrosis Education Conference, which will be held July 25 to 27, 2025, at the Ameswell Hotel in Mountain View, CA. Registration for the Conference is open.

These promising projects are made possible through generous contributions from the community.
Thank you to all whose support is advancing innovative cystic fibrosis research.

MAY IS CYSTIC FIBROSIS AWARENESS MONTH

During Cystic Fibrosis Awareness Month, we honor our community while educating others about CF and our ongoing need to find a cure.

CF Purple Power CF Awareness Challenge:
Purple is the color of CF awareness. Help us turn social media purple to raise awareness and understanding of cystic fibrosis while supporting CFRI!

Post your favorite purple-themed photos and videos, tag us, and challenge your friends to donate and join in! This year you are not limited to purple hair - here are some ideas:

— ***Purple hair, nails, makeup or beard***
— ***Purple outfits or accessories***
— ***Purple foods or drinks***
— ***Purple sunsets, pets, pet gear, home décor etc.***

The possibilities are endless!

Each day of May, CFRI will release a CF-related fact; share with your friends! Every post spreads awareness. Every donation supports CFRI's programs.

https://curecf.cfri.org/PurplePower

Go purple!

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 person you 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.

DONATE LIFE
www.donatelife.net

In Honor of

September 16, 2024 — March 31, 2025

Alexander Adams
Lindsey Bachtel
The Baim Family
Bridget Barnes
Lucy Barnes
The Baugh Family
The Behm Family
Allison Bertagnoli
Allison Best
Robert Boswell
Brian Burks
Gabriela Castillo
John Christie
Mickey Conway
Lauren Colonna Cooper
Cameron Cornell
Weston Covak
Barbara and Jim Curry
Stacy Dean
Chuck and Edna Devore
Gordon DeVore
Mackenzie Dondanville
Tess Dunn
Meg Dvorak
Nancy E
Janelle and Andre Estournes
Alanah Rosenbloom Fink
Victoria and Oscar Flamenco
The Frisbee Family
Zaylee Fults
Sean Gallagher
Cheri Geoghegan
Elyse Elconin Goldberg
Gianna Gutierrez-Serrato
Gwen
Sonya Haggett
Brendan Harrigan
Melanie Henshaw
Niall Hibbard
Susan Lane Hoffman
Courtney Hollis
Anna Modlin Holyoak
Leanne Ibarra
Michelle Jones
Waylon Jones
Joni
Joy
Cary and Ralph Kelly
Jeremy Kharrazi
Franny Kiles
Kay Kinney
Kristin Favero Konvolinka
Santosh Krishnan
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Michael Livingston
Joseph Arthur Lopez
Emily Fredrick Lucas
Markiplier
David Martin
Jean Mathews
Rachael and Rebecca McMullen
Carly McReynolds
Caleb Mercer
Jonathan Miller
Hannah Mitchell
Angela Morey
Jim and Alice Morrison
Austin Murray
Bill Mylar
Jessica Nett
Pamela Nett
Samuel Olszta
Tristan O'Neill
Aly, Maddie and Killian O'Reilly
Molly Pam
Colin Penuel
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Robyn Primack
Michael Reuscher
Megan Reveles
Rebecca Roanhaus
Carl Robinson
Elizabeth Rogers
Taylor Rolefson
Nancy S
Linda Sanford
Corey Sarkis
Ella Grace Schultz
Sebastian Sharpe
Janice Shaul
Shelly
Mike and Carol Shrout
Wende Sinnaeve
Kandra Leigh Smith
Alma St. Lawrence
Mckaylin and Alayna Stoddard
Beth Sufian
Sandy Sufian
Melisa Compton Sullivan
Brian Tacke
Spencer Tacke
Ian Terrell
Adam Thompson
Katherine Thompson
The Tirumala Family
Robert Turk-Bly
Ricky Whicker
Ella Whitcomb
Jonathan Witczak
Kate Yablonsky
Laura Zellmer

In Memory of

September 16, 2024 — March 31, 2025

Tony A
Gordon Adelman
Sonya Lyn Akister
Gianna Rose Altano
Joe Anderson
Gary Anderson
Jessica Arvidson
Debbie Babbitt
Ann and Ronald Baldwin
Cara Baysinger
David Beebee
Anne and Irvin Beltrame
Kathleen Beltrame
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Jerome and Aurore Vaeth
Cindy Vidak-Haley
Joy Villasenor
Tom Walton
Nicky Wastell
Tara Weir
Carol Wilmott
Claire Wineland
Josiah Woodhouse
Shawn and Scott Zavodjancik

2025 CF Retreat Program: Created By and For Adults with CF

CFRI's Retreats for Adults with cystic fibrosis bring together community members from across the nation and globe. Our recent virtual Spring Retreat in March was attended by over 50 adults with CF as well as family members and friends. Our annual CF Summer Retreat will be held in person at the Jesuit Retreat Center in Los Altos, California from August 14 to August 19 (with a virtual component). The Retreat offers adults with CF a welcoming place to engage with their peers. For those attending in person, we will have our usual stringent cross-infection and COVID-19 protocols in place. For those unable to travel to California, many of the sessions will also be offered online.

The retreat will provide health-related and psychosocial support programs and activities including:

- Exercise activities;
- Educational workshops with guest speakers from across the country;
- Rap sessions (support groups) to support positive mental health;
- Dedicated time to connect, network, and socialize with others.

Attendees consistently report that the retreat offers new information about CF research and therapies, dramatically improves psychosocial health, and provides resources and strategies for coping with the daily challenges of CF. While the retreat is geared toward adults with CF, attendees may invite adult family members and supportive peers.

This year, the CF Adult Retreat Planning Committee is selling an array of clothing items, with all proceeds supporting the Retreat. The striking design is available in multiple colors and styles. To shop, go to **https://curecf.cfri.org/LoveHeals2025**.

Join us! For more information, go to **www.cfri.org** or call Mary Convento at **650.665.7559**.

The CF Retreat is generously sponsored by Vertex Pharmaceuticals, AbbVie, and private donors.

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

Those impacted by cystic fibrosis (CF) experience higher rates of depression and anxiety than the general population. With its unpredictability, daily treatment burden, and diverse symptoms, CF is a challenging disease for those diagnosed, as well as for those who love them. Studies show that depression and anxiety can negatively impact adherence to one's medical regimen. In response, CFRI offers a range of programs to address the psychosocial and mental health needs of our community.

Counseling Support: CFRI provides up to $125 per session for six sessions of counseling to individuals with CF (children and adults), and their family members with the licensed provider of their choice in their community. Participants must live in the U.S.

Support Groups: CFRI offers nine monthly online support groups for our diverse CF community members. Participants register once and then are able drop in monthly to connect with their peers. All groups are facilitated by professionals who work in CF centers or trained individuals who have CF themselves.

Monthly groups are offered for:

- *Caregivers/Parents of Children with CF;*
- *Caregivers/Partners/Spouses of adults with CF;*
- *Adults with CF;*
- *Adults with CF Post-Transplant;*
- *Adults with a Late CF Diagnosis;*
- *Teens with CF;*
- *Spanish-Speaking CF Community Members;*
- *Those Who have Lost a Loved One to CF;*
- *Adults with CF Who Are Ineligible/Cannot Use Modulators*

Mindfulness: Mindfulness has been shown to reduce anxiety and depression. In 2025, CFRI is offering four quarterly workshops, each focused on different aspects of mindfulness and how it can offer positive coping skills for those living with CF. "Mindfulness to Manage Pain," took place in March. The next session, "Mindfulness to Cope with Fear and Anxiety," is scheduled for Tuesday, June 10. Open to those with CF and their family members, the workshops are taught by Julie Desch, MD, who herself lives with CF.

All groups and workshops are held via Zoom, and participants log in from across the country and world. Please refer to our website for monthly meeting dates.

These programs are offered at no charge to our community members, thanks to our supportive sponsors. For more information, visit our website, or email Sabine Breon at sbreon@cfri.org.

Partners in Living Initiative – CF Quality of Life Programs are generously supported by Viatris, Vertex Pharmaceuticals, the Boomer Esiason Foundation, Genentech, and private donors, as well as contributions through CFRI's CF Quality of Life Program, a Living Legacy of Peter and Kathy Judge.

Aging in the New Era of Cystic Fibrosis

Continued from Cover

by adults with CF who shared the impact of the specific session's topic on their lives.

The program identifies, educates, and connects the next generation of multidisciplinary CF care providers and thought leaders, while serving as a catalyst for innovative research activities. Fellows were deeply impacted by their participation in the program. As one wrote in a post-symposium evaluation, "The opportunity for multidisciplinary discussion in such an intimate setting was astounding. I left the conference with so many ideas for future areas of research, and I can't remember the last time I felt this inspired. I also very much valued the opportunity to meet physician scientists of incredible caliber and experience; the idea that I would be able to collaborate with these individuals is incredibly exciting." Another wrote, "This was truly the most incredible and rewarding opportunity I have had in my career."

A key element of the program's impact was the matching of fellows to faculty mentors, who generously share their time to provide insights and guidance as the fellows pursue activities and new lines of research related to the care of adults with cystic fibrosis. In accepting their place in the program, fellows committed to participating at quarterly post-symposium informational meetings. These sessions advance fellows' knowledge, while strengthening the cohort bond and future collaborative efforts.

2024 Patrick Nash fellows, faculty, and steering committee members

Based on the program's success in its inaugural year, 2025 training program details are being finalized. The agenda is set, the 2025 faculty recruited, the location in Chicago secured, and the online application open. With each year, the number of clinicians and researchers who have participated in the Fellows Training Program will grow. Representing multiple disciplines, this national network will advance critical research while developing improved care models for our aging cystic fibrosis community. Patrick Nash would be very proud.

Patrick Nash Fellows Training Program Steering Committee

— Steven Freedman, MD, PhD
— Richard Moss, MD
— Carolyn Nash
— Christine Nash, MBA
— Anna Payne*
— Ahmet Uluer, DO, MPH
— Siri Vaeth, MSW
— James Yankaskas, MD

* *We remember our beloved committee member Anna Payne, who lost her battle with stage 4 colon cancer in early 2025.*

Patrick Nash Fellows Training Program Faculty

— A. Whitney Brown, MD
— Steven Freedman, MD, PhD
— Richard Moss, MD
— Joseph Pilewski, MD
— Deepika Polineni, MD
— Melissa Putnam, MD
— Jennifer Taylor-Cousar, MD, MSCS
— Ahmet Uluer, DO, MPH
— James Yankaskas, MD

CFRI thanks Viatris, Vertex Pharmaceuticals, and the family of Patrick Nash for the grants and donations that made the 2024 program possible.

2025 Mothers' Day Fundraiser Brings Awareness and Hope!
CFRI and the CF Community Need Your Support!

CFRI's annual Mothers' Day Celebration fundraiser is an annual tradition that involves hundreds of people, promotes awareness of cystic fibrosis (CF) among our friends and family around the globe, and ultimately raises needed funds to support CFRI's vital services to the CF community.

Your participation will have a meaningful impact!

While we celebrate therapeutic advancements that provide better health to many living with CF, thousands of people with CF are unable to benefit from the new CFTR modulators. We still have no cure for CF and the median age of death remains far too low.

This card's artwork, "Love #18 of 65 Roses," was painted by Wanda Olson, who lost her 33-year-old daughter Michele to CF in 1999. Inspired by the story of a child who misheard his diagnosis of cystic fibrosis as "sixty-five roses," the rose symbolizes hope for a cure for CF. Wanda is creating 65 rose paintings in Michele's memory.

There are several ways to send your Mothers' Day invitations: in the mail, online or through Facebook. If you have questions, or need assistance, please contact Mary at **650.665.7559** or **cfri@cfri.org**; or go to **http://cfri.org/mothers-day-celebration/**.

Thank you for supporting CFRI and those living with cystic fibrosis!

Many Voices ~ One Voice: Advocating for Our Community

At the regional, state and federal level, CFRI engages our cystic fibrosis community to raise awareness of the ongoing physical, financial and emotional burdens of the disease. These include the lack of a definitive cure, the impact of its status as a rare disease, the need for increased CF research funding, limitations of newborn screening programs, and barriers faced in accessing care. Through its Many Voices ~ One Voice Cystic Fibrosis Advocacy and Awareness Program, CFRI provides tools and opportunities for our community members to share their experiences with decision makers, so as to improve health and quality of life.

CFRI remains engaged with rare disease coalitions to create state Rare Disease Advisory Councils. The definition of a rare disease is one that impacts fewer than 200,000 people in the United States. Cystic fibrosis is one of 10,000 identified rare diseases that impact 1 in 10 Americans. State Rare Disease Advisory Councils (RDACs) provide the community with a formal platform and official voice to help advise state officials on policies and services that impact us. Currently, 29 states have RDACs.

The growing number of co-pay accumulator policies is an ongoing concern. These policies do not allow payments from drug manufacturer assistance or other programs to be applied toward a person's deductible and total out-of-pocket expenses and create significant financial hardship for many members of our community. A recent study found that 39 out of 50 states have at least one insurance plan with a copay accumulator adjustment policy (CAAP). CFRI is working with others to support state and federal legislation that would ensure that all payments for prescriptions be applied toward one's deductible and annual out-of-pocket total.

CFRI follows many issues and weighs in on those that impact our CF and rare disease communities. These include proposed Medicaid cuts (over 55% of children with CF are covered through state Medicaid programs); supplemental oxygen reform; coverage for cancer early detection screening; and Pharmacy Benefit Manager (PBM) reform. We champion improvements to newborn screening programs, which often miss members of our community with less common mutations, while raising awareness and providing resources related to the elevated risk of cancer faced by those with CF.

There is no shortage of ways to get involved with CFRI's advocacy efforts. ***Join us!***

Supported to date through grants from Vertex and Genentech.

A Hybrid Gene Correction Strategy for Cystic Fibrosis

Continued from Cover

Our group has successfully optimized the process of editing upper airway basal cells using ex vivo editing techniques. However, engrafting these cells back into the airways remains a challenge. A potential solution is to adjust the delivery technique to enable direct mutation correction within the lung tissue itself. Therefore, we developed an approach to evaluate viral and non-viral delivery reagents in precision-cut human lung slices (PCLS), a model of lung tissue. This ex vivo platform maintains the native cellular architecture and composition of the human lung, providing a physiologically relevant system to assess the efficacy and safety of various gene editing delivery methods for people with CF.

There are two components of the gene correction system that we need to deliver for successful correction: the CRISPR/Cas9-sgRNA, a nuclease that creates a cut in the DNA and the donor template to be used by the cell to correct the genetic defect. We evaluated various delivery strategies including lipid nanoparticles (LNPs), virus-like particles (VLPs), lentiviral vectors (LVs), adeno-associated viral vectors (AAVs) and cell-penetrating peptides (CPPs). Each delivery system offers distinct advantages and limitations for targeting the basal cells (stem cells of the lung) in PCLS, with considerations including packaging capacity, cellular tropism and editing efficiency.

Selective organ targeting (SORT) LNPs, designed to specifically target the lung, delivered nucleic acids to nearly all primary Human Bronchial Epithelial Cells (HBECs) in vitro (>99%). Moreover, SORT LNPs carrying Cas9 mRNA and a previously validated sgRNA targeting the CFTR gene resulted in 78% editing in CF HBECS. Correction of the most common CF mutation was achieved by combining these SORT lipid nanoparticles with an AAV donor vector. We found that using this hybrid approach we were able to correct up to 73% of alleles in CF HBECs. The correction efficiency could be further enhanced by using AZD7648, an inhibitor of the Non-Homologous End Joining pathway, achieving up to 81% corrected alleles. We are now validating this therapeutic approach in precision-cut human lung slices.

As expected, delivery directly to an intact tissue model where the 3D-structure, the extracellular matrix and cell type heterogeneity of the lung is preserved has been challenging. However, SORT LNPs can deliver nucleic acids to up to 34% of cells within lung slices. Delivery is possible across different cell types including basal cells, goblet cells, ciliated cells and alveolar cells. We hope to be able to present updated results at CFRI's Conference in July 2025. These findings represent an important step toward developing effective in vivo gene editing approaches for people with CF, demonstrating the potential of SORT LNPs to access diverse cell populations within the complex architecture of lung tissue. While we are excited about our progress, we also recognize that this is just one potential strategy to the challenge of correcting the underlying mutations that cause cystic fibrosis and we are proud to be part of the portfolio of approaches that the CFRI supports. We share the belief that through this broad portfolio we will develop transformative cures for people with cystic fibrosis.

CFRI Is Your Partner in Living

- **PURPLE POWER CF AWARENESS CHALLENGE:**
  Challenge friends and family to help us turn social media purple to raise CF awareness and support CFRI. Post your pics of purple outfits, purple makeup, purple food, etc. and tag us!
- **HOLD YOUR OWN VIRTUAL EVENT:**
  Cocktails for a cure, a benefit yoga session, Pictionary challenge – no idea is too big or too small. Create an event, and we'll help you make it happen.
- **FACEBOOK:**
  Every penny raised through Facebook goes to CFRI with no fees. Many community members create fundraisers for CFRI by donating their birthdays or other special events on Facebook.
  Go to **https://www.facebook.com/cfri.curecf**, scroll down to Fundraisers, and click on **Create!**
- **MONTHLY GIVING:**
  Champions of Hope! Donations to *Champions of Hope* provide a consistent revenue stream to support research to find a cure for CF and enhance CFRI's programs in CF education, support and advocacy. To participate, go to our website or contact Stacie Reveles (see below).
- **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.
- **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.
- **PLANNED GIVING:**
  Benefits provided through planned giving may include increased income, substantial tax savings, opportunity to meet your philanthropic goals, and the satisfaction of making a very significant gift to CFRI 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.
- **VEHICLE DONATIONS:**
  CFRI is partnering with CARS (Charitable Adult Rides & Services) to accept donations of used vehicles. CARS takes care of everything from the pick-up and sale to sending you the donation receipt and tax documents. You can donate a car, motorcycle or boat.

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

SAVE THE DATES!

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

CF Virtual Support Groups

See groups and dates on page 4,
or go to **www.cfri.org**
for information.

All support groups are free
and held on Zoom.

Mindfulness Workshops

June 10, 2025
Mindfulness and Fear/Anxiety

September 9, 2025
Mindfulness and
Sadness/Depression

December 9, 2025
Mindfulness and Peace/Joy

Quarterly online Mindfulness
workshops for the CF community
with Dr. Julie Desch

CFRI 38th National CF Education Conference

July 25 – July 27, 2025

Ameswell Hotel, Mountain View, CA
and Online

See back page for speakers,
topics, and sponsors

CF Summer Retreat - Hybrid

August 14 – August 19, 2025
Jesuit Retreat Center,
Los Altos, CA
and Online

CFRI's Gala "A Breath of Fresh Air"

Saturday, October 11, 2025
Hillsborough Racquet Club
Hillsborough, CA

For information or to register for these events, please email cfri@cfri.org or call 650.665.7559.

SAVE THE DATES!

CFRI Cystic Fibrosis Research Institute
RESEARCH · EDUCATION · ADVOCACY · SUPPORT

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CFRI's mailing list is confidential. We do not sell our list, nor do we give out any names or addresses under any circumstance.

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, Viatris, AbbVie, *and* **Arna Pharma**

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

CFRI's 38th annual Conference

will launch on **Friday, July 25,** with presentations by CFRI-funding researchers, by renowed clinician-researchers, and by members of our CF community.

Exhibitors, our new "Innovations and Libations" reception, the annual awards dinner, a dance party, and interactive workshop with world-renowned artist Dylan Mortimer, make this a full weekend of community connection.

Virtual attendees can view all presentations live on our interactive event platform.

In-Person Registration: $240 – *includes all presentations, conference meals, award banquet, receptions, and support groups.*

Single Day Rates Available: $55 – $150 **Virtual Registration:** ***Free***

Room rate at the boutique Ameswell Hotel only $149 per night – *must book through CFRI block (use QR code on the right or link on our Conference website).*

Ameswell Hotel

Confirmed speakers to date include: Anais Amaya, PhD • Nadia Ameen, MD • Benjamin Chan, PhD • Jaelyn Cooper, MHS • Steven Freedman, MD, PhD • Raksha Jain, MD • Steven Jonas, MD, PhD • Ron Kopito, PhD • Kaitlyn Kortright, PhD • Meghan McGarry, MD, MAS • Dylan Mortimer, MFA • Matthew Porteus, MD, PhD • Gregory Ratti, MD • Celeste Riepe, PhD • Ruby Sims, PhD • Nicole Tovar, PT, DPT • Ahmet Uluer, DO, MPH • Daria Van Tyne, PhD • Katrine Whiteson, PhD • Feng Yuan, PhD

For more information, visit **www.cfri.org** or call **855.cfri.now**.

Conference Info.

Generously sponsored to date by: **Vertex Pharmaceuticals, AbbVie, Viatris, ReCode Therapeutics,** *and the* **Boomer Esiason Foundation**

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