

## CFRI's 36th National Cystic Fibrosis Education Conference: Presentation Abstracts

CFRI held its 36th National CF Education Conference, Hope on the Horizon, as a hybrid event July 28 - 30, 2023. Over three days, attendees from across the country and globe heard from nationally-renowned speakers in a wide range of CF-related topics. These presentations are now available for viewing on CFRI's YouTube channel: <https://tinyurl.com/tbjzwm4y>. The abstracts below appear in a shortened version.

### A Journey Through Rare: Because EVERYONE Deserves More Tomorrows

Rachel Alder  
Salt Lake City, UT

Rae Alder is a fierce advocate and has been since her early childhood years. Subsequently, Rae has crafted a life's work centered in the art of advocacy and authenticity. In her moving presentation, Rae shares her personal timeline of navigating the world of rare. Rae overcame years of racial bias, health disparities, and rapid health deterioration to finally receive the correct diagnosis of cystic fibrosis in January 2023, at the age of 26. During her moving presentation, Rae includes a timeline of her symptomatology, along with pivotal life experiences that



subsequently led her to the world of patient advocacy for everyone facing cystic fibrosis.

### Phage Therapy and Cystic Fibrosis

Saima Aslam, MD, MS  
University of California, San Diego, CA

Bacteriophages (phages) are ubiquitous in the environment and are present within the

human microbiome as well. Phage therapy constitutes the usage of lytic-only phages targeted to specific pathogens with clinical intent. Several successful cases of phage therapy in the setting of cystic fibrosis were discussed including its use in multidrug resistant *Pseudomonas aeruginosa*, *Burkholderia*

*Continued on page 13*

## Mechanism of Intracellular Persistence of *Pseudomonas aeruginosa* in CF Epithelial Cells



Naren Gajenthra Kumar, PhD, Suzanne Fleiszig OD PhD — Herbert Wertheim School of Optometry and Vision Science, University of California, Berkeley, CA

Infections with the bacterial pathogen *Pseudomonas aeruginosa* (*Pa*) are often associated with poor prognosis and quality of life for individuals with CF. While drugs like inhaled tobramycin are routinely used to treat infections with *Pa*, they do not target bacteria that hide inside epithelial cells that line the airways. In cell culture experiments we have shown that bacteria inside cells can be restricted to smaller compartments called vacuoles. Once in this location, they can resist treatment with antibiotics such as ofloxacin

*Continued on page 12*

## CFRI Community

Fall 2023

**Editorial Chief**  
Siri Vaeth, MSW

**Editors**  
Bridget Barnes  
Sabine Brants, MA  
Zoe Davies, NP

**Contributing Writers**  
Sabine Brants, MA  
Bill Hult  
Siri Vaeth, MSW  
36th Annual National Education  
Conference Presenters

**Layout and Design**  
Marina Michaelian Ward

**Board of Directors**  
Bill Hult, President  
Jessica Martens, Vice President  
Zoe Davies, NP, Secretary  
Oscar Flamenco, CPA, Treasurer  
Francine Bion  
Julie Desch, MD  
Colleen Dunn, MS, RRT, CCRD  
Elyse Elconin Goldberg  
Jean Hanley, MD  
Doug Modlin, PhD  
Richard Moss, MD  
Arek Puzia, CPA, MBA  
Ahmet Uluer, DO, MPH

**Executive Director**  
Siri Vaeth, MSW

**Programs & Finance  
Supervisor**  
Mary Convento

**Programs & Outreach  
Associate**  
Sabine Brants, MA

**Advocacy & Programs  
Associate**  
Stacie Reveles

**Social Media Associate**  
Danielle Mandella

**Programs & Development  
Assistant**  
Leeya Kannankunni

CFRI Community is published  
and distributed to friends  
of CFRI for free.

Send address corrections and  
other correspondence to CFRI:  
1731 Embarcadero Road  
Suite 210, Palo Alto, CA 94303  
Phone: Toll Free  
855.cfri.now (855.237.4669)  
Fax: 650.404.9981  
Email: cfri@cfri.org  
Website: www.cfri.org

With our permission, you may  
reproduce original information  
from this newsletter with credits to  
CFRI Community and the author.

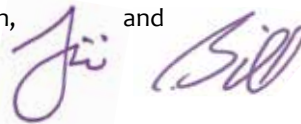
## Letter from the Executive Director and President of the Board

Dear CFRI Community,

We hope that you are safe and well. This fall, CFRI celebrates 48 years of research funding and service to our community. We owe a debt of gratitude to that small group of parents who first formed this enduring and responsive organization. At that time, life expectancy for those with CF was very short. Through the years, we have witnessed many exciting advances in the field. While we celebrate the transformative impact of CFTR modulators upon many members of our community, we recognize that many members of our community remain without new therapies. We will not rest until everyone – regardless of CFTR mutation – has needed therapies, and ultimately a cure.

Thanks to your support this past year, we increased the number of funded research grants, while expanding our education and support programs. As a member of our community, you play a key role in bringing innovative research and programming to life. With your help we continue to move closer to a cure.

Peace and good health,



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



## From Copay Accumulator Programs to Skin Cancer: 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 skin cancer, oral health considerations, running with cystic fibrosis, conference presentations, and more. Some podcast episodes feature inspirational stories, from Rise up for a Dream with Nick Kelly to CF in Pakistan – A Doctor's Crusade for Change. 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 Viatrix.

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

In 2023, two retreats were held for mothers of children and adults with CF. The first weekend in May, CFRI's 9th annual Embrace Mothers Retreat was offered in-person at Vallombrosa Retreat Center, attended by 23 mothers from eight states. The virtual retreat was held on September 30th and attended by 25 mothers from 10 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.

Since its inception, Isabel Stenzel Byrnes led the writing workshops at the Embrace retreats. She led her final Embrace workshop at the May retreat. It is now hoped that the powerful pieces created through the years will be collected and published in a book of writings, and dedicated to Isabel.

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 participants in a writing workshop led by Isabel Stenzel Byrnes, LCSW, MPH*

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 Retreats for Adults with CF Keep the Community Connected

Last August, CFRI offered its annual retreat for adults with CF as a hybrid event. Nearly 70 people registered, with one-fourth attending in person at Vallombrosa Retreat Center in Menlo Park, California, and others attending from around the nation and globe. The Retreat provides a welcoming community for adults with CF looking for connection, information, and camaraderie with their peers.

The retreat provided a wide array of health-related and psychosocial support programs and activities. In addition to exercise activities tailored to individuals' unique capacities, participants heard from experts in the field. 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. Mark your calendars for the virtual Spring Retreat, held March 15 – 17, 2024.

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

# 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.
  - **NEW! Late Diagnosis Support Group:** This group is offered to adults who had a late CF diagnosis. Meetings are held on the first 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](http://www.cfri.org), or email Sabine Brants at [sbrants@cfri.org](mailto:sbrants@cfri.org).

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





Nadia Ameen, MD



Benjamin Chan, PhD



Carlos Milla, MD



Kenichi Okuda, MD, PhD



Zachary Sellers, MD, PhD



Katrine Whiteson, PhD



Paul Bollyky, MD, PhD



Kevin Qingquan Chen, PhD



Matthew Porteus, MD, PhD



Anais Amaya Colina, 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 nine researchers. Much of this research will benefit all those with CF, regardless of their CFTR mutation.

## New Horizons Award Program:

- **Nadia Ameen, MD, Principal Investigator**  
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 (CF)*
- **Benjamin Chan, PhD, Principal Investigator**  
Yale University School of Medicine  
*Optimization of Phage Therapy to Reduce Pseudomonas-Induced Inflammation in 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*
- **Zachary Sellers, MD, PhD, Principal Investigator**  
Stanford University  
*Targeting IRBIT to Correct Bicarbonate Secretory Defects in Cystic Fibrosis*
- **Katrine Whiteson, PhD, Principal Investigator**  
University of California Irvine  
*Targeting recalcitrant CF pathogens with phages, antibiotics, and small molecule adjuvants*

## Elizabeth Nash Memorial Fellowship Program (For Post-Doctoral Fellows):

- **Paul Bollyky, MD, PhD, Principal Investigator**  
**Kevin Qingquan Chen, PhD, Postdoctoral Fellow**  
Stanford University  
*Targeting Bacterial Resistance to Phage Therapy in Cystic Fibrosis*
- **Matthew Porteus, MD, PhD, Principal Investigator**  
**Anais Amaya Colina, PhD, Postdoctoral Fellow**  
Stanford University  
*A Hybrid Gene Correction Strategy for Cystic Fibrosis*

## Special Circumstances Grant:

- **Beate Illek, PhD, Principal Investigator**  
University of California San Diego  
*CFTR mRNA Delivery to CF Airways*



Beate Illek, PhD

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



CF Wellness Program was developed in recognition of the positive impact of movement and exercise upon one's physical and mental health. These online classes 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.

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 classes, from Yoga and Groov3 to stretching 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 either the night before or the day of each class.

No experience is required for any classes, and all abilities and mobilities are welcome! For the complete schedule and to register, go to [cfri.org/wellness-classes/](https://cfri.org/wellness-classes/).

CFRI's CF Wellness Classes are sponsored by Vertex Pharmaceuticals and Viatris, with additional support from individual donors



## Faces of CF: Serving our Diverse Community

Imagine suffering every symptom of cystic fibrosis for years, but your physicians do not test you for the disease because the color of your skin does not match their incorrect assumptions about race and CF. 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. 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, whose input is vital in the creation of enhanced outreach and support.

CFRI recently conducted its second annual survey of CF social workers, asking them to identify gaps in resources experienced by their patients, and in particular, those patients and families who do not speak



English. The top four cited resources were financial support; printed resources; online videos and podcasts; and support groups. We will use this information to build upon progress made in 2023.

CFRI has greatly expanded our print resources, including the updated "Fibrosis Quística en la Clase," while ensuring our website is

fully accessible in multiple languages. Our YouTube channel has a growing list of podcasts available in Spanish, and we offer an online monthly support group for Spanish-speaking members of our community, facilitated by a licensed clinical social worker.

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.

CF can be an isolating disease. 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.

CFRI's Faces of CF Diversity & Inclusion Program is sponsored by Viatris, Vertex Pharmaceuticals, Gilead Sciences, Genentech, and Chiesi USA

# CFRI's A Breath of Fresh Air Gala A Success!

CFRI's annual gala was held on October 14, 2023 at the beautiful Hillsborough Racquet Club. In-person guests enjoyed the festive atmosphere, delicious food and fine wines. The gala program launched with an inspiring video message from Nicholas Kelly, while our emcee, Chris Chmura of NBC Bay Area, guided us through a fast-paced but moving program which highlighted CF community members and CFRI-funded researchers. Arek and Marta Puzia, parents of a young daughter with CF who cannot benefit from current CF modulator therapies, stressed the importance of continued investment in CF research. We honored Ann Robinson, MA, former Executive Director of CFRI and CF Community Mentor, as our 2023 Cystic Fibrosis Champion. The program ended with a moving musical performance by Tess Dunn, a singer and writer who lives with CF. The gala was livestreamed and virtually attended by community members across the country.

By the end of the evening, over \$120,000 was raised to support CFRI's research, education and support programs. \$40,000 of this total was matched dollar-for-dollar by members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle and designated for our CF research awards.

Warm thanks to our generous sponsors, in-kind donors, attendees and hardworking Gala Committee members. Everyone played a role in our gala's success – it was truly *A Breath of Fresh Air!*

**A Breath of Fresh Air Sponsor** — Vertex Pharmaceuticals


**Mistral Sponsors** — AbbVie; Chiesi USA

**Zephyr Sponsor** — Nestlé Health Science



**Breeze Sponsors** — Gainsight; Heritage Bank of Commerce; InterWest Insurance Services LLC; Longfellow Real Estate Partners; Viatrix

**Media Sponsor** — NBC Bay Area



## 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 Fundraising Event:** Cocktails for a cure, yoga, cornhole, pickleball – 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 and more – 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 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.

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

# Many Voices ~ One Voice: CFRI's Advocacy & Awareness Efforts on Behalf of the CF community

Through its *Many Voices ~ One Voice* Cystic Fibrosis Advocacy and Awareness Program, CFRI engages our CF community to raise awareness of the burdens and complications of the disease, including the lack of a cure, impact of its rare disease status, the need for CF research funding, and barriers faced by our community in accessing care.

Cystic fibrosis is a rare disease, defined by the federal government as one that impacts less than 200,000 people in the nation. It is estimated that 1 in 10 Americans has a rare disease. CFRI is engaged in efforts to create state Rare Disease Advisory Councils (RDACs), which provide the community with a formal platform and official voice at the state level to help advise state officials on policies and services that impact us. Currently, half the states in the U.S. have RDACs, and CFRI is a member of several coalitions working to advance these efforts.

CFRI also participates with several coalitions to advance legislative bans on co-pay accumulator programs, which do not allow payments from drug manufacturer assistance to be applied toward a person's deductible and total out-of-pocket expenses. These programs are now embedded in 64% of private insurance plans and create significant financial hardship for many members of our community. CFRI is working with others to support legislation mandating 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 nineteen states and Puerto Rico, with broad bipartisan support. On the federal level, bipartisan legislation has been introduced in both the House and Senate (H.R.830 and S.B.1375, the Help Ensure Lower Patient Copays Act (HELP Copays Act)). If you would like to get involved, or need more information, please contact us at CFRI.



Supported through grants from the Bucks County CF Alliance, AbbVie, Vertex Pharmaceuticals, Genentech, and Gilead Sciences



## 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](http://www.cfri.org) to make a donation online.



# In Honor of

March 15, 2023 — September 30, 2023

Gordon Adelman	Dylan Dunn	The Hoyt Family	Carly L. McReynolds	Corey Sarkis
The Adelman Family	Sharon Dunn	Alexander Jenkins	Jonathan Miller	Janice Shaul
Bridget Barnes	Tess A. Dunn	Barbara M. Jensen	Matthew Mitchell	Kandra Leigh Smith
Lucy Larkin Barnes	Janelle and Andre Estournes	Jimmy and Elizabeth	Harper and Juliet Murphy	Kate Smith
Erin Barzanty	Jarrold Fischer	Darren Johst	Jessica Nett	Ethan Spain
The Baugh Family	Kathleen Flynn	Laine Jones	Pam Nett	The Thibault Family
Ryan Bortz	Zaylee Fults	Michelle Jones	Lindsey Jensen Nijmeh	Adam Thompson
Robert Boswell	Cheri Geoghegan	Franny Kiles	Tristan O'Neill	Robert Turk-Bly
Linda Burks	Mark E. Gerow	Eleanor Kolchin	Scott Parks	Heidi Umber
Naomi Burks	Emma Ghione	Santosh M. Krishnan	Hema Patel	Maria Cristin Urruela
Shaun Collins	Larissa Giuliano	Steven Kusalo	Briauna Peters	Siri Vaeth
Mary Convento	Elyse Elconin Goldberg	Joseph Librers	Megan Reveles	William Vogt
Lauren Colonna Cooper	Sonya Haggett	Emily Fredrick Lucas	Rebecca Roanhaus	Devin Wakefield
Cameron Cornell	Treyvon M. Hall	Marc Antony Maciel	Ann Robinson	Scott Wakefield
Jordan Cote	Niall Hibbard	Larissa Marocco	Carl Robinson	Kassi Watkins
Barbara and Jim Curry	Jeremiah Holdaway	Eric Marten	The Robinson Family	Melissa Weiner
Bradley Daniel	Courtney Hollis	Rose Logue Martini	Taylor Rolefson	Nina Wine
Chuck and Edna DeVore	Erinn Hoyt	Elizabeth A. Mayer	Alanah Fink Rosenbloom	Jonathan Witczak
Gordon DeVore	Kristen Hoyt	Rachael and Rebecca	Grayson and Peyton	
Ann Du Frane	Scott Hoyt	McMullen	Rudnick	

# In Memory of

March 15, 2023 — September 30, 2023

Chelsa Aboud	Neva L. DeVore	Mary Kay Jones	Kim and Scott Nelson	Joseph M. Sinnaeve
Carol, Marcus and Kimberly Adelman	Vicki Dippner-Robertson	Peggy B. Jones	Michele Denise Olson	Doris Sprugasci
Gianna Rose Altano	Xuan Doan	Kathy and Peter Judge	Jennifer Ortman	Anabel Stenzel
Mary Andrade	James Ekegren, MD	Kurt Koenig	Dellene Ott	Betty Jane Strole
Jessica Arvidson	Susie Ellerson	Peggy and Janet Kessell	Larry Otter	David, Laurie and Norma Stuckert
Jennifer Bartolomucci	Robert Faller	Edward Kinney	Rose Patane	Erin Phillips Taylor
David Beebee	Veronica Juliet Fleming	Lori Kipp	Lisa Pearne	Tara Telford
Anne C. Beltrame	Judy, Yvonne and BJ Florenza	Mike Koslow	Shirley Quick	The Thibault Family
Jamie Bertolini	Jessica A. Fredrick	Jane Ellen Kulik	Catherine Rawlings	Roxanna Thomas
Debbie Boswell	Carol Fuller	Nancy T. Lane	Ronald Reed	June Thompson
Rebecca Boyer	Laura Gale	Joyce Levine	Dea Roanhaus	John Trask
Greg Brazil	Wallace Erby George III	Lauren Levine	Katie Robinson	Louis Anthony Trigueiro
Alicia Brogle	Norma Gibson	Michelle Libby	Rob Robinson	Todd Trisch
Kyle Butler	Ray Goldstein	Sean Linehan	Pamela Rockhold	Jerome Vaeth
Ryan Butler	Brian J. Gower	Dawn Longero	Thomas Rockhold	Rory and Jerry Vaeth
Isabel Stenzel Byrnes	Will Harbison	Jennifer Longoria	Tom Rolefson	Tom Walton
John Carpenter	Cherri S. Harris	Alyson Lowery	Lawrence Ross	Sean Waltrip
Ryan Coelho	Nicholas Hollis	Nu Ly	Dave Ross	Angela Watkins
George and Martha Colla	Robert Hunziker	Bob and Nadine Mackey	Mary Roy	Tara Weir
Deborah A. Cox	Christopher Ireland	JoAnn Mahaffey	Suzanne Ruff	Kelly A. Wilson
Parker G. Cronin	Janice	David McAfee	Dhea Schalles	
John Cunningham	Brian Jensen	Nichole McMillan	Timothy Schenck	
Caroline Daly	Edward Jensen	Jessica Mobley	Jeffery Allen Seale	
		Kimberly Myers	Linda Sherry	

# Honoring Our Community Heroes

At the 36th 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.



## David Stuckert Memorial Volunteer of the Year Award — Zoe Davies, NP, PNP

Zoe Davies is a Pediatric Nurse Practitioner who for nearly three decades was a pivotal member of the CF Research Team at the Stanford Center for Excellence in Pulmonary Biology. Zoe was passionate about teaching, supporting, and providing the best care to patients who participated in CF clinical trials. Over the past 30 years Zoe earned the trust and affection of colleagues, patients, and families. For years, Zoe has been a dedicated volunteer with CFRI, where she serves as Secretary of the Board of Directors and on the CFRI Community Newsletter Committee.



## CFRI CF Professional of the Year Award — Deepika Polineni, MD, MPH

Deepika Polineni is Director of the Cystic Fibrosis Center and an Associate Professor of Pediatrics at Washington University in St. Louis, Missouri. Her research program focuses on the identification of non-CFTR genetic modifiers of CF lung disease using human airway transcriptomics and metabolomics to identify novel gene targets, and airway cellular models to study mechanisms of influence. These efforts are aimed at improving care for people with CF and advancing new therapies, particularly mutation-agnostic nucleic acid-based treatments, with the goal of reducing health disparity gaps and serving a global CF community. Dr. Polineni gives generously of her time to support the broader CF community, including serving on CFRI's Medical Advisory Committee and Research Advisory Committee.



## CFRI Partners in Living Award

### in Memory of Anabel and Isabel Stenzel — Scott Pinner, MD

Scott Pinner is a husband, father, athlete, and Physical Medicine and Rehabilitation physician. He also lives with cystic fibrosis. As a youth, Dr. Pinner maintained his lung function through sports, but after the rigors of medical school, residency, and launching his medical career, his health declined in his late thirties. He was listed for transplant, but prior to receiving this, he endured colon cancer with serious complications and numerous pneumothorax. He received a life-saving double lung transplant in 2014; since then, he has become a strong advocate for organ donation, and an athlete in the Transplant Games. A long-time member of the CFRI community, he currently serves on CFRI's Research Advisory Council.



## Paul M. Quinton Cystic Fibrosis Research

### Legacy Award — Dieter Gruenert, PhD (Posthumous award)

Dieter Gruenert was a geneticist in the Department of Otolaryngology-Head and Neck Surgery at the University of California San Francisco, where he developed many of the human CF and non-CF airway epithelial cell lines used in airway disease research throughout the world. Dr. Gruenert developed a prototype of targeted genome editing called Small Fragment Homologous Replacement (SFHR) which paved the way to more efficient CRISPR/Cas9 method. His group focused their attention on gene editing approaches using induced pluripotent stem cells (iPSCs) in order to develop novel therapeutic strategies for inherited diseases. He was the recipient of many CFRI Research Awards. Tragically, Dr. Gruenert passed away unexpectedly in 2016. His legacy continues, as his groundbreaking research led to new breakthroughs in the field of cystic fibrosis.

## 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

# Community Abounds at the 39th Annual Golf Tournament for CFRI

On August 14, 160 dedicated golfers gathered for the 39th annual Cystic Fibrosis Benefit Golf Tournament at Cinnabar Hills Golf Club in San Jose. Participants enjoyed 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 2024 will be announced soon!



## 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.



## SAVE THE DATES!

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

### Zoom Into Now

October 24 • November 28  
December 26 • January 23  
February 27 • March 26

Monthly online Mindfulness and meditation sessions for the CF community with Dr. Julie Desch.

### CF Virtual Support Groups

See dates on page 10

### Retreat for Adults with CF

March 15 – 17, 2024

A virtual retreat for adults with CF, their friends and family.

### Purple Hair Challenge

May 2024

Color your hair purple and challenge your friends to raise CF awareness!

### Embrace Mothers' Retreat

May 3 – 5, 2024

An in-person retreat for mothers of children and adults with CF.

Vallombrosa Retreat Center  
Menlo Park, CA

### CFRI 37th National CF Education Conference

July 26 – 28, 2024

A hybrid event

### CF Summer Retreat for Adults with CF

August, 2024

More details coming soon!

For information or to register for these events, please email [cfri@cfri.org](mailto:cfri@cfri.org) or call 650.665.7559.

### Mechanism of Intracellular Persistence of *Pseudomonas aeruginosa* in CF Epithelial Cells

*Continued from Cover*

that get inside cells. With the support of CFRI, our recent work studied how intracellular bacteria develop resistance to ofloxacin by identifying novel genes expressed by resistant populations and detailing the protein composition of the vacuole where bacteria persist.

Our results show that vacuolar bacteria able to resist antibiotic treatment reduce Type III toxin gene expression and increase expression of genes possibly involved in the import of metabolites abundant in the CF environment. Deleting one such group of genes resulted in increased clearance of vacuolar bacteria after antibiotic treatment only in cultured CF epithelial cells and not normal bronchial epithelial cells. We also show that vacuoles, where surviving intracellular bacteria persist, are decorated with a host protein specific for an acidic compartment whose function is to degrade intracellular bacteria. The involvement of the acidic environment in resistance to ofloxacin was confirmed when changing the intracellular pH from acidic to neutral made intracellular bacteria more susceptible to antibiotic treatment. This suggests



the acidic environment of the vacuole may be involved in the adaptation of intracellular bacteria to antibiotics.

With a focus on therapeutic interventions, these findings can inform future studies that target vacuolar bacteria and reduce the burden of persistent *Pseudomonas* infections in individuals with CF. A better understanding of the vacuolar compartment, where bacteria reside, will help leverage existing methods and develop novel strategies to target and treat intracellular *Pseudomonas* infections. Lastly, the adaptation of bacteria to, or the protection provided by the vacuolar environment suggests the involvement of a yet unknown mechanism of evading antibiotic treatment and will be subject of future work.

## CFRI's Biennial Community Survey: *Arriving in your Inbox this Fall*

CFRI encourages our community to participate in our biennial survey, which provides our community members the opportunity to share what CFRI programs are most meaningful to them, while helping us to identify emerging issues and unmet needs. Please help us to continue our responsive programming! Your voice is important, and we thank you for your engagement and participation.



Rachel Alder

Saima Aslam

*ceenocepacia complex* and *Mycobacterium abscessus* infections. The phage therapy clinical experience at UC San Diego was reviewed, where they have a success rate of almost 80% in antibiotic recalcitrant infections. A variety of factors play a role in the clinical effectiveness of phage therapy including the type of phage used, multiplicity of infection, synergy with other phages and/ or antibiotics, concentration of phage used, mode of administration, stability of the clinical preparation, development of serum neutralization, and type of infection treated, among others. In general, phage has been found safe for clinical use. In the presentation, Dr. Aslam discusses the basic concepts of phage therapy, and ongoing research in the area of cystic fibrosis and multidrug resistant pathogens, including clinical trials in this field.

### All Hands of Deck to Cure Cystic Fibrosis

Matthew Porteus, MD, PhD  
Stanford University, Palo Alto, CA



Matthew Porteus

In the original formulation of genome editing, it meant that one could change the sequence of the genome just as one could change a document on your computer. Recently, genome editing has been most broadly used to make gene knockouts to directly fix genetic variants that cause disease. Yet, direct correction of mutations is ultimately the most powerful approach to transforming medicine for those with genetic diseases. The most versatile approach to

gene correction is using genome editing by homology directed repair (HDR). Using HDR we can correct a wide variety of disease-causing mutations using a single strategy. We have applied this approach to developing a one-size fits all approach for the thousands of mutations that cause cystic fibrosis. The HDR approach works best in engineering cells outside the body (“ex vivo”). We have used genome editing to correct cystic fibrosis causing mutations in basal cells (stem cell of the airway) and now demonstrated that those cells can repopulate the epithelium of the sinus in mice. The presentation also discusses the next steps needed to obtain FDA clearance to test the approach to treat serious sinus disease in cystic fibrosis patients.

### Understanding and Managing Pain in CF: A Biopsychosocial Approach

Amanda S. Bruce, PhD and  
Deborah Friedman, PhD  
University of Kansas Medical Center,  
Kansas City, KS; Massachusetts General  
Hospital CF Center, Boston, MA



Amanda S. Bruce

Deborah Friedman

Pain affects up to 75% of children and 89% of adults with CF. It can have a negative impact on mood, quality of life, and overall physical functioning, and can interfere with an individual’s ability to engage in treatments for CF, school, work, and daily life activities. Treatment for pain ideally involves a multi-component approach that may involve both medication and non-medication interventions. A specific non-medication approach to pain management, called cognitive-behavioral therapy (CBT), has been shown to be effective for reducing pain and its interference in daily life with a low risk of side effects in other chronic diseases. CBT is also effective in treating depression, anxiety, and sleep problems that often co-occur with pain.

In this presentation we provide an overview of a research study we are conducting to

develop a telehealth, CF-specific mind-body program for pain management that draws from well-established CBT and acceptance-based approaches. Our multi-center research team has demonstrated feasibility, and effectiveness of a similar CF-specific 8-session CBT program for the prevention and treatment of depression and anxiety. CF care team members at centers across the U.S. and Canada are being trained to provide this program to patients as part of routine CF care. Our pain management study will follow a similar model by eliciting input from the CF community and CF care team members to develop a pain management program that addresses the specific needs of people with CF. In our presentation, we discuss our findings from stakeholder interviews about the experience of living with both CF and chronic pain, as well as describe evidence-based mind-body approaches to pain management.

### From Defining Health Disparities to Improving Health Equity in Cystic Fibrosis

Susanna A. McColley, MD, FAAP, ATSF  
Ann & Robert H. Lurie Children’s Hospital  
of Chicago, Chicago, IL

The term “health disparity” is used to describe a difference in health, in which a specific characteristic leads to better or worse health in a population. “Health inequity”



Susanna A. McColley

is a difference in health that results from unfairness and injustice. Health disparities have been reported in cystic fibrosis for decades. For example, people with CF from lower socioeconomic status (those with fewer financial resources) have worse health outcomes across continents and countries with different health care, insurance, and CF care systems. There are also worse health outcomes in people with CF from minoritized racial and ethnic groups, even when taking socioeconomic status into account. Both are also true in many other common and rare diseases. In CF, scientific and public health advances have made a major impact on the health of the CF population but have widened disparities.

*Continued on page 14*

## CFRI's 36th National Cystic Fibrosis Education Conference

Continued from Page 13

In this presentation, we discuss how CF clinical trial enrollment, newborn screening, and global availability create inequities. We also discuss how policies and practice must advance so that every person with CF has a fair and just opportunity to attain their highest level of health.

### Panel: Parenting with CF

Lucy Barnes; Matthew DeFina; Carl Robinson  
Ashland, OR; Napa, CA; Danville, CA  
Moderated by Mary Helmers, RN  
Lucile Packard Children's Hospital Stanford,  
Palo Alto, CA



Lucy Barnes



Matthew DeFina



Carl Robinson

Improved treatments for cystic fibrosis – most notably CFTR modulators – and the resulting increased life expectancy have translated to larger numbers of individuals with CF becoming parents. Speakers on this engaging panel share their individual paths to parenthood, including adoption, in vitro fertilization (IVF), and pregnancy / childbirth. Moderated by Mary Helmers, who has counseled many of her patients with CF about available options as they weigh the decision to become parents, the discussion explores panelists' range of experiences related to reproductive health discussions with their CF care providers; their partners' key roles; finding balance with parenting, working and managing CF; the impact of pregnancy and / or parenthood upon health maintenance; and addressing psychosocial care needs.

## Advances in mRNA Therapy: New Applications for Cystic Fibrosis

Deepika Polineni, MD, MPH  
Washington University School of Medicine,  
St. Louis, MO



Deepika Polineni

It is estimated that up to 10% of people with cystic fibrosis (CF) in the United States are unable to benefit from currently FDA-approved CFTR modulator therapies due to their CFTR mutations (i.e., genotype) or a history of side effects to such treatments. Research is underway for alternative strategies to improve the health of people with CF who do not benefit from CFTR modulators. Messenger ribonucleic acid (mRNA) therapy is one such treatment option that is under investigation for people with CF and has often recently been included under the term "genetic therapies" for CF. Importantly, mRNA therapy is distinct from gene therapy and gene editing. mRNA is a type of ribonucleic acid that is present in human cells and represents one step in the process of the DNA genetic code becoming translated into a functional protein. Using mRNA replacement therapy as a treatment in CF involves the careful delivery of mRNA coding for CFTR into airway cells to use the cells' own machinery to create normal CFTR protein in the lungs. The success of this depends on many factors including the stable maintenance and delivery of the therapy.

Recently, mRNA was utilized successfully in development of mRNA-based SARS CoV-2 vaccines for COVID-19 during the pandemic. Lessons learned from these vaccines and the pandemic will continue to inform investigations of mRNA in therapeutic development and use. mRNA therapy is now under early phases of study for safety and tolerability in people with CF who cannot benefit from CFTR modulators based on their CFTR mutations. mRNA could also have broader future applications for people with CF independent of their CFTR mutations. In summary, mRNA therapy in CF is a new treatment under investigation that could have potential to improve lung disease for people with CF irrespective of their CFTR genotype. This presentation provides a review of mRNA as a novel therapeutic option.

## Embracing the Future: Aging with CF

Ahmet Uluer, DO, MPH  
Boston Children's Hospital/ Brigham &  
Women's Hospital, Boston, MA



Ahmet Uluer

Cystic fibrosis (CF) is a multisystem disorder primarily affecting the respiratory and digestive systems. Advancements in treatment, including highly effective modulators, have significantly improved quality of life for individuals with CF.

The incidence of cancer involving the GI tract occurs at higher rates for people with CF than the general population, particularly those receiving organ transplant. Regular screening and surveillance are essential to detect and not only manage but even prevent malignancies. Individuals with CF are at an increased risk of developing diabetes and over 35% have this listed as a diagnosis. Monitoring for elevated blood pressure also important, especially for those with diabetes. Furthermore, people with CF are also at risk of hearing loss and kidney disease associated with life-saving treatments. Monitoring and screening measures are important to prevent and manage both kidney disease and hearing loss. If necessary, interventions involving hearing aids and cochlear implant can impact quality of life. Cardiovascular disease is also emerging as a concern among adults with CF. Regular cardiovascular screening, including monitoring of lipid profiles and assessment of cardiac function, is necessary to identify and manage cardiovascular complications promptly. Early identification and aggressive management are essential to mitigate their impact on overall health and well-being.

As the adult CF population continues to grow, it is imperative to recognize the complications associated with aging in individuals with CF. Screening, early treatment, and understanding of effective management strategies are essential to improve the quality of life for adults living with CF.

## CF and Colon Cancer: My Lived Experience

Anna Payne  
Langhorne, PA



Anna Payne

Anna Payne is a 36-year-old cystic fibrosis and colon cancer patient. She is also the founder of the Bucks County CF Alliance, a non-profit organization. In her talk, she describes her experience with CF and living with stage-4 colon cancer. She was diagnosed at the age of 34, well before the recommended screening age of 40. Those with cystic fibrosis have 5 to 10 times the rate of colon cancer as the general public: those post transplant have over 25 times the risk. After her diagnosis, Anna immediately focused her advocacy efforts on educating as many people as possible about the elevated risk of developing colon cancer experienced by CF patients and carriers. She details her story, from diagnosis until today; what she has learned and what we can do to help make sure this doesn't happen to others.

Presenting alongside Dr. Ahmet Uluer, Anna shares the personal side of this two-part medical story: the real-life impact that colon cancer has on people and what it looks like living with two diseases.

## Advances in Stem Cell Research for the Treatment of CF

Brigitte Gomperts, MD  
University of California, Los Angeles, CA



Brigitte Gomperts

This is a very exciting time in CF research. The first clinical trial of nebulized CFTR mRNA airway delivery has been completed and several more clinical trials are in the pipeline. While we await the results

of these trials, research is moving forward with even more advanced gene therapy approaches. But central to understanding how gene addition or gene editing will work

for CF is knowing which cells in the airway should be targeted. In this presentation, we share our recent findings using single cell RNA sequencing of the multitude of different cell types and subtypes in the human airway and their function. We also discuss how these cell subtypes change in CF. We focus in on which cell types express CFTR and examine the different airway stem cells. Airway stem cells are of particular interest because gene editing of these cells could potentially lead to long-lived correction of CFTR.

We review the current approaches to replace, repair or restore the CFTR gene in the airway, which include mRNA delivery, gene delivery, gene editing, and cell therapy approaches. Cell therapy approaches are in their infancy, and we explore the different kinds of stem cells that could potentially be used and the pros and cons of each stem cell type. There are several barriers to airway delivery of these gene therapeutic approaches which are even more challenging in CF, such as thick, tenacious mucus and inflammation. There are also barriers to systemic gene delivery and gene editing in the body. Cell therapy approaches have their own delivery issues and the additional hurdle of engraftment in the airway. We discuss each of these barriers and potential ways to overcome these obstacles with advances in delivery systems. Overall, we show major progress in the field of gene therapy for CF that is being made through cutting edge research in this area and provides hope that this could provide a therapeutic approach for all CF patients.

## Normalizing the Abnormal

Alanah Rosenbloom, MSW  
San Jose, CA



Alanah Rosenbloom

Navigating a life with CF is tough... to say the least! Having CF can feel weird, gross, and unpredictable, especially when all you yearn for is to be normal and in control. It has taken Alanah decades to gain

some perspective on the struggle that is living with CF, and while she doesn't necessarily feel normal or in control, she does feel incredibly empowered by the experience

thus far. After living with CF for 37 years, she is ready to share some of her stories.

Join Alanah as she talks about: the funny, the bad, and the ugly; befriending others with CF; using CF to be of service to others. Whether you have CF or not, Alanah hopes this talk generates hope that what once brought shame can one day become cherished.

## Conference Sponsors & Exhibitors

### CFRI Recognizes Our Generous Sponsors and Exhibitors For Their Support of the 36th National Cystic Fibrosis Education Conference

#### Premiere Sponsors —

Viatrix; Vertex Pharmaceuticals

#### Sustaining Sponsor —

Genentech

#### Diamond Sponsors —

Gilead Sciences; Chiesi USA

#### Platinum Sponsors —

AbbVie;

Boomer Esiason Foundation

#### Bronze Exhibitors —

4DMT; Alcresta Therapeutics;  
Digestive Care, Inc.; First Wave Bio;  
Foundation Care, An AcariaHealth  
Solution; NeilMed;  
Walgreens Specialty Pharmacy

#### Organizational Exhibitors —

Cystic Fibrosis  
Engagement Network (CFEN);  
Emily's Entourage; UC San Francisco

#### Supporter —

Prodigy Press, Inc.



1731 Embarcadero Road • Suite 210  
Palo Alto, California 94303

Change Service Requested

Nonprofit  
Organization  
U.S. Postage  
PAID  
Palo Alto, CA  
Permit #24

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

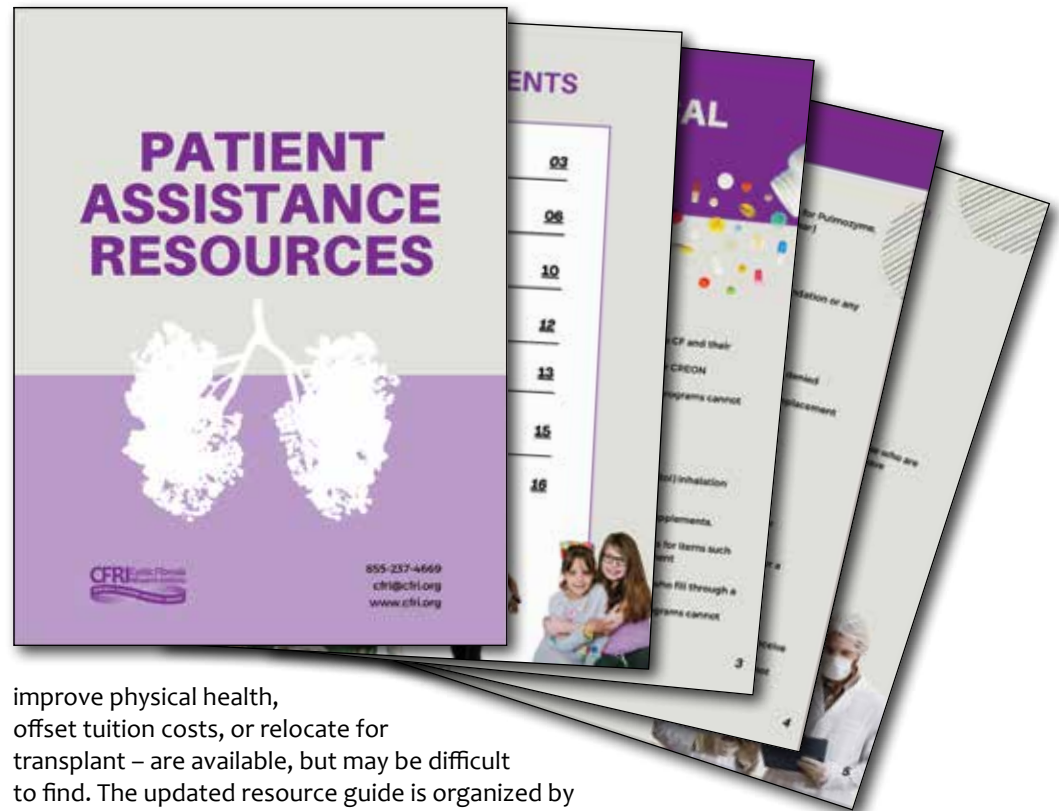
To find a cure for cystic fibrosis while enhancing quality of life for the CF community.

For their generous support of CFRI Community, special thanks to:  
Vertex Pharmaceuticals,  
Gilead Sciences,  
AbbVie, Chiesi USA,  
and Viatrix

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

## CFRI's Community Resource Guide: Available in English and Spanish

CFRI's resource list has been updated to help our CF community members connect to resources. Financial challenges are significant for many members of our community. A 2019 study conducted by the Milken Institute School of Public Health found that one in three people living with CF faces food insecurity. Diverse forms of assistance – to access medications,



improve physical health, offset tuition costs, or relocate for transplant – are available, but may be difficult to find. The updated resource guide is organized by type of assistance and available in Spanish and English on our website ([www.cfri.org](http://www.cfri.org)). This is a living document: if you have resources to share, please contact us – so that we may update and maintain an up-to-date guide.

Cystic Fibrosis Research Institute a 501(c)(3) nonprofit organization Federal EIN# 51-0169988

