

Aging With CF: Special Considerations Siri Vaeth, MSW

I spent my entire life wanting to grow older. And now I'm over 50 and my body is showing normal but challenging signs of aging with CF. Menopause, aches and pains, bone loss, poor eyesight... I try to remember these are all the benefits of still being alive!"

— Isabel Stenzel Byrnes, LCSW, MPH,
living with CF and lung transplant recipient

Advances in cystic fibrosis (CF) therapies have steadily increased survival for those living with the disease, and many members of our CF community are now reaching middle age. Until recently, issues that impact all people as they age – cancer, heart disease, diabetes, arthritis – were often not discussed by medical care providers with their CF patients. The improved health of many people with cystic fibrosis – especially among those benefiting from highly effective modulator therapies – has moved these age-related issues to the forefront. Growing old with CF presents its own unique blessings and challenges. The resilience required to live with CF is further tested with age.

Pulmonologists at adult CF centers are now encouraging broader health screenings and the integration of preventative care. Says Dr. Ahmet Uluer, director of the Adult CF Center at Brigham and Women's Hospital and Boston Children's Hospital, "Current guidelines do not adequately address the changing landscape of aging during the highly effective modulator era. We urgently need a modified lens to expand

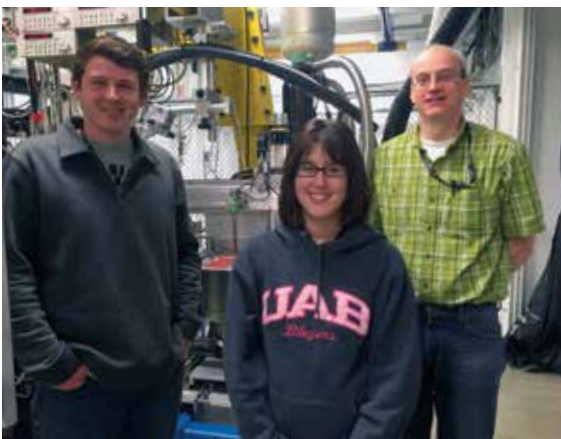


and adjust the age of screening, identify and prevent other complications, and support age-related CF research."

CF and Cancer

Individuals with cystic fibrosis have five to ten times the rates of colon cancer as the general population. For those post-transplant, who are dependent upon

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Cole Martin, Dr. Christina Le and Dr. Stephen Aller

Role of CFTR Arginine-933 in Channel Function and Drug Potentiation

*Wei Wang, Lianwu Fu, Cole L. Martin, Steven M. Rowe and Stephen G. Aller
University of Alabama at Birmingham*

Cystic fibrosis is caused by protein defects in the CF transmembrane conductance regulator (CFTR). CFTR is an inherently difficult protein to study because: 1) it is located in the cell membrane; 2) folding of CFTR from non-CF individuals is still inefficient compared to other membrane proteins; 3) many mutations in CFTR that cause disease fold extremely inefficiently, and 4) it is very difficult to achieve sufficient folded/functional protein for biochemical studies. Even today, very rare mutations of CFTR, such as changes to Arginine-933 (Arg933),

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Letter from the Executive Director

Dear Friends,

CFRI has continued to expand its programs due to our community's evolving needs. CFRI is an organization that is by and for the CF community. Launched in 1975 by a group of parents of children with CF, today every level of the organization – including the Board of Directors, staff, and committees – is comprised of members of our community. Adults with CF, CF family members, researchers and clinicians all bring unique perspectives and expertise. The result is a mission-driven and responsive organization that unites people impacted by CF from around the nation and globe.

We pursue our mission thanks to our generous and compassionate organizational partners, sponsors, and community members. Our research, advocacy, education, support, and wellness services are dependent upon the generosity of our community.

CF remains a harsh and capricious disease. Over 10% of our community members cannot use CFTR modulators, and people of color are overrepresented in this group. Lung transplant recipients need better therapies. And as those with CF age, a host of new issues emerge; while celebrating expanded longevity, we are focused on new strategies for care and support.

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

Warmly,



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



News from the Board

Dear CFRI Community,

I hope that you are safe and well. As President of the Cystic Fibrosis Research Institute's Board of Directors, it is an honor to be a member of such a responsive organization and engaged community.

CFRI was able to sustain its robust research funding and programmatic offerings in 2022, despite the economic challenges of inflation and the ongoing pandemic. This was possible due to the strong support of our donors and sponsors, as well as the efforts of CFRI's staff, which operates with tremendous efficiency. We remain attentive to the evolving needs of our community and are grateful for your commitment and the spirit of partnership that enables us to meet new demands.

The funding of cutting-edge research remains key to our progress, and I am delighted to share that in 2023 we will be providing grants to nine CF researchers. CFRI's Research Advisory Committee and Board of Directors will soon select the 2023 projects to fund; we look forward to sharing the full list of researchers in June. As a member of CFRI's community, you are playing a role in advancing science.

Thank you for your ongoing support. With your help we will continue to move closer to a cure, while enhancing the lives of those living with cystic fibrosis.

Peace and good health,



Bill Hult | President, CFRI Board of Directors



The Many Faces of Cystic Fibrosis

CFRI is committed to inclusion, justice, and equity for all members of the cystic fibrosis (CF) community. CF impacts people of every race and ethnicity, and our Faces of CF Diversity & Inclusion Program raises awareness of this among medical care providers and communities of color. Our work is guided by an engaged CF Diversity and Inclusion Advisory Committee, whose input is vital in enhancing CFRI's outreach, resources, and support.

CFRI recently hosted a webinar for CF care team members to explore unconscious bias and its impact upon cystic fibrosis patients and caregivers. Titled, "Navigating Bias: Lessons Learned from Lived Experience," the event featured three members of the CF community – Alicia Maciel, Jaelyn Cooper and Marianela Fajardo – as well as CF social worker Kimberly Morse. Their often painful experiences with bias as patients, caregivers and providers were shared. Importantly, this webinar provided concrete tools for addressing unconscious bias, with the goal of improving communication between care teams and patients that can translate to improved outcomes.

Jaelyn Cooper, who serves on CFRI's Diversity and Inclusion Advisory Committee, shared her experiences as a Black woman with CF. Diagnosed at 18 months, Jaelyn

recounted her mother's determined advocacy for testing to determine why Jaelyn was so sick. Shared Jaelyn, "Just because of the color of my skin, that shouldn't be the reason that this (CF) is ruled out. Every symptom was there that indicated CF." As an adult, Jaelyn has needed to emulate her mother's advocacy skills, especially during hospitalizations when she is seen by doctors who are not part of her CF care team. During one stay, the physician reviewed her chart and was in disbelief that she had CF, noting it is a disease impacting white people. Jaelyn shared, "I felt not heard and also frustrated. We come to the hospital looking for healing and care and understanding and hope and instead... I have to be on guard or almost in defense mode or ready for battle, which is not how it's supposed to be."

Experiences like this are shared by many members of the CF community. Outcomes for people of color with CF remain worse than those of white patients, with the



CF Foundation reporting that Black and Hispanic people with CF have a nearly two-fold higher risk of death before the age of 18. CFRI is working to raise awareness of these issues and CFRI is committed to addressing disparities in diagnosis, care and health outcomes for people of color with CF.

CFRI's Faces of CF Diversity and Inclusion Program is supported through educational grants from Viatrix, Vertex Pharmaceuticals, Genentech, Gilead Sciences, and Chiesi USA.

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

CFRI's 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.

This year, CFRI offers classes in a new format. In order to increase variety and accessibility, 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 track and/or the Saturday track, and attend as many or as few 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/.

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



Aging with CF: Special Considerations

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immunosuppressants to prevent organ rejection, the risk increases to 25 to 30 times that of the general population.

Currently, the Cystic Fibrosis Foundation recommends that those with CF have their first colonoscopy at age 40 (30 for those post-transplant), but there are many who believe that this age should be lower. “I was diagnosed with stage 4 colon cancer at the age of 34,” says Anna Payne. “Colon cancer symptoms mimic CF-related GI symptoms. I would never have survived until 40 to get that first screening. I strongly believe the age must be lowered.” Adds Dr. Uluer, “People with CF live with the expectation the other shoe is going to drop. Health screening such as colonoscopy can not only detect but prevent the other shoe from dropping by potentially preventing colorectal cancer.”

Skin cancer is another common cancer, particularly for those post-transplant. Regular screening by a dermatologist and a daily commitment to the use of sunscreen and protective clothing are the best defense. In addition to colon and skin cancers, rates of pancreatic, small bowel and other GI cancers are elevated for those with CF. For those post-transplant, the risk of developing cancer increases dramatically, with studies finding increased rates of lip, cervical, testicular, kidney, lung, and bladder cancers, and lymphomas. The importance of regular screenings cannot be overstated.

Heart Disease

Heart disease among those with CF was historically associated with chronic hypoxemia (low levels of oxygen in the blood), and was most often seen in those with advanced lung disease. This is beginning to change since the approval of the triple-combination modulator Trikafta. Many who struggled for decades to keep weight on now find themselves with significant weight gain and obesity, leading to a higher risk of hypertension or high blood pressure.

CF-Related Bone Disease

It is very common for osteopenia to develop in adolescence, and adults with CF have higher rates of osteoporosis. Reasons for this include CFTR dysfunction that leads to lower bone mineral density, poor absorption of calcium, vitamins D and K, the use of glucocorticoids, CF-related diabetes, and



delayed nutrition. Individuals with CF should have their first DEXA scan (bone density scan) by age 18 to detect bone loss and prevent fractures.

Hearing Loss

The use of IV antibiotics to treat pulmonary exacerbations has played a key role in improving survival. If not appropriately monitored, and even when they are, some of these antibiotics may be associated with ototoxicity, inner ear damage that develops as a side effect of the medications. Often beginning with a ringing in the ears (tinnitus), ototoxicity can advance to severe hearing loss and balance issues. Hearing loss is associated with depression, social isolation, and cognitive decline. Hearing tests should be routinely conducted to monitor the impact of specific IV and inhaled medications.

Liver Disease

As our CF community ages, the prevalence of CF-related liver disease is growing. The mucus that is the hallmark of CF may also block bile ducts in the liver. When bile cannot exit the liver, inflammation and scarring occurs, and the liver cannot function properly. Long-term use of IV antibiotics can also damage the organ. While there is no specific test for CF-related liver disease, clinical assessment of symptoms, blood tests and ultrasounds are used to make a diagnosis.

Menopause

It has long been understood that hormones can play a role in lung function and exacerbations. As more women with CF reach the age of menopause, a new field of research has launched to assess the impact of menopause upon women with CF. The CF Foundation notes that women with CF may enter menopause two to three years before women in the general population. Menopause has impacts upon bone loss, lung function, muscle strength and mental

health, and the management of symptoms should be explored with one's CF care team. The Cystic Fibrosis Reproductive and Sexual Health Collaborative (CFReSHC) has an excellent resource on CF and menopause which can be found at cfreshc.org.

Dental Health

Studies have found that individuals with CF are more likely to have a lower pH in their saliva, and reduced saliva secretion. This can be worsened by medications, and can lead to oral infections, gum disease and cavities. While addressing dental health may have been a lower priority for many in light of addressing lung infections and other CF complications, as CF community members age, there is growing recognition of the need to monitor and support dental health.

Mental Health

The TIDES study demonstrated the higher rates of depression and anxiety experienced by those living with cystic fibrosis. Growing old with CF can create an entirely new source of fears, anxiety and depression. Financial concerns may increase for people who never expected to survive to an age where a retirement fund would be needed. New age-related health complications and the isolation created by cross-infection risks exacerbate anxiety. Lower energy or cognitive changes can make one's self-care management overwhelming, leading to weariness. The correlation between mental and physical health is direct. It is as important to address one's mental health as one's physical health. Counseling, support groups, social engagement, mindfulness practices, and physical activity are all strategies that can support improved mental health. Having access to disability resources and affordable insurance programs as people with CF age is critical as well.

As we celebrate the medical advances that have led to an aging cystic fibrosis community, we must also recognize and address the interplay of CF with the challenges of age-related health issues. Shares Isabel Stenzel Byrnes, “When I was 22, my 46-year-old friend and mentor with cystic fibrosis said, ‘Aging ain’t for sissies.’ Wasn’t he correct? But it sure beats the alternative. Aging gracefully with CF invites us to remain tenacious, informed, and grateful.”

Special thanks to Ahmet Uluer, DO, MPH, and Isabel Stenzel Byrnes, LCSW, MPH, for their assistance with this article.

CFRI Advocacy and Awareness: About Copay Accumulator Programs

By Siri Vaeth, MSW

Through its Many Voices ~ One Voice Cystic Fibrosis Advocacy and Awareness Program, CFRI engages our community to raise awareness about the burdens of cystic fibrosis (CF), the impact of its rare disease status, the diversity of our community, the critical need for research funding, and barriers that impede access to therapies and care. One such barrier to care is the proliferation of copay accumulator programs that are embedded in nearly two-thirds of private insurance plans nationwide.

Copay accumulator programs impose significant financial hardship upon patients. Many CF community members use copay assistance from drug manufacturers and charitable organizations to access their medications. Historically, these funds were applied toward a person's deductible and out-of-pocket expenses. Once the full value of the assistance was utilized, patients would usually have met their deductible and be able to afford their copay.

With a copay accumulator program, the payers accept the funds from the copay assistance, and, after the copay assistance is fully expended, force the patient to meet their deductible. In essence, the deductible is paid twice: once by the manufacturer or charity, and once by the patient.

Those who rely on specialty medications are most impacted by these policies. While some argue that copay assistance is a strategy by pharmaceutical companies to drive people to expensive name brand drugs instead of generics. The vast majority of copay assistance is used for prescriptions that have no generic equivalents. This is certainly true in the cystic fibrosis community.

Studies have shown that copay accumulator programs impede access to therapies and exacerbate health disparities. When out-of-pocket prescription costs reach \$75 to \$125, more than 40% of patients leave their medication at the counter. When these costs reach \$250, over 70% of patients

will forego their medications. This leads to greater risks of health decline and costly emergency room visits.

To date, 16 states and Puerto Rico have passed laws to make all copays count; another dozen states have legislation in the works. This includes California, where CFRI has joined the ALS Association, Hemophilia Council of California and the California Rheumatology Alliance to sponsor AB 874 to ensure all copays count. At the federal level, HB830 has been introduced to ban copay accumulator programs. This legislation has tremendous bipartisan support.

There are many ways to get involved with CFRI's advocacy and awareness efforts. We seek your engagement! Please email Stacie Reveles at sreveles@cfri.org for more information.

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

CFRI Funds Innovative CF Research

At the core of CFRI's mission is to pursue a cure for cystic fibrosis through supporting innovative research. CFRI is committed to ensuring that research will continue toward a cure. 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. We seek to attract the next generation of researchers and foster a long-term thriving national CF research community, with an emphasis on outreach to ensure gender, race and ethnic diversity. We also seek to encourage research that will contribute toward therapies for those ineligible for CFTR modulators.

Members of CFRI's Research Advisory Committee (RAC) are currently in the process of reviewing and vetting a diverse range of high-quality research proposals. The RAC recommendations will guide CFRI's Board of Directors, which will vote on 2023 research grant awards, with awardees to be shared with our CF community in early May.

Current CFRI-funded researchers include:

Elizabeth Nash Memorial Fellowship Award:

- Suzanne Fleiszig, PhD, Principal Investigator; Naren Kumar, PhD, Post-Doctoral Fellow; UC Berkeley – *Characterizing the intracellular diversification of Pseudomonas aeruginosa in chronic lung infections.*
- Paul Bollyky, MD, PhD, Principal Investigator; Nina Pennetzdorfer, PhD, Postdoctoral Fellow; Stanford University – *Targeting Bacterial Resistance to Phage Therapy in Cystic Fibrosis.*

New Horizons Award

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

The researchers above will present their work at CFRI's National Cystic Fibrosis Education Conference on Friday, July 28, at the Grand Bay Hotel San Francisco in Redwood City, CA.

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 Hair Challenge:

Purple is the color of CF awareness! If you were ever tempted to dye your hair and you want to raise CF awareness, now is the time to go purple! Join us, and challenge your circle of friends and family to support our CF community. It's simple, and follows the idea of the ALS ice bucket challenge.

Step 1 — Accept the Challenge! Color your hair purple (all or a portion) with permanent or temporary dye, or use a phone app to do this digitally!

Step 2 — Post a photo or video of your purple locks on your social media network with the hashtag #purplehairchallenge; tag us and/or make a donation to CFRI.

Step 3 — Challenge others to dye their hair, and/or donate to the cause by tagging them and sharing this link:

<https://give.classy.org/cfri-purple-hair-challenge>

Go purple!

Sponsored by
Vertex Pharmaceuticals and Chiesi USA.



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.



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October 16, 2022 — March 15, 2023

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October 16, 2022 — March 15, 2023

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2023 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 over \$110,000 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 only 34.

The artwork on this year's card was painted by Wanda Olson, mother to Michele, who passed away at the age of 33 from CF complications. 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. To honor her daughter's memory, Wanda is creating 65 unique rose paintings. The beautiful cards feature the 35th painting in the series – a Double Delight Rose.

No matter how you would like to send your Mothers' Day invitations – in the mail, online or through Facebook - you can contact



Mary at 650.665.7559 or cfri@cfri.org to assist you; or go to <http://cfri.org/mothers-day-celebration/>. Thank you for supporting CFRI and those living with cystic fibrosis!

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 \$120 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 monthly online support groups for our diverse CF community members. Participants are able to register once and then drop in monthly to connect with their peers. All groups are facilitated by Licensed Clinical Social Workers who either work in CF centers or 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;
- Teens with CF;
- Spanish-Speaking CF Community Members;
- Those Who have Lost a Loved One to CF.



Mindfulness – “Zoom Into Now:” These monthly online sessions combine mindfulness practices with meditation, which has been shown to reduce anxiety and depression. Open to those with CF and their family members, 16 years and up. Taught by Julie Desch, MD, who herself lives with CF.

All groups are held via Zoom, and participants log in from across the country and world. Please refer to the “Save the Dates” on page 11 or our website for 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 Brants at sbrants@cfri.org.

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

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 Horizon and Elizabeth Nash Memorial Fellowship CF research awards.

Please join this inspiring group! Become a member of the 2023 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. Challenge Circle Members receive a certificate, CFRI swag, and updates on our research awards.

Our Circle was initiated by Suzanne Freiley, whose beloved niece, Jessica Fredrick, lost her battle with CF at the age of 21. Despite tremendous progress in CF therapies, we continue to lose treasured members of our



community to this cruel disease, and there is still no cure.

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.

2022/2023 CF Research Challenge Circle Members (as of 3/28/2023)

Britton and Vahna Benedict
John and Sallie Best
Francine and Joel Bion
Rhonda and Bernie Fredrick
The Galper Family
Martha Keller
Stephen and Julie Kimball
Kathy and Michael Konkel
in memory of Jason Konkel
Beth and Dan McMullen
Fred A. Miller III
Doug and Robin Modlin
Christine Nash and Andrew Chen
Elizabeth Nash Foundation
Wanda Olson
Devin Wakefield
And donors who wish to remain anonymous.

2023 CF Summer Retreat: Created By and For Adults with CF August 18 – 22, 2023

CFRI's Retreats for Adults with CF bring together community members from across the nation and globe. Our recent virtual Spring Retreat was attended by 50 adults with CF as well as family members and friends. Now we are gearing up for our annual CF Summer Retreat, which provides a welcoming community for adults with cystic fibrosis looking for connection and camaraderie with their CF peers. We hope to hold the 2023 Summer Retreat in person at Vallombrosa Retreat Center in Menlo Park, California. We will have stringent cross-infection and COVID-19 protocols in place.



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

- Exercise activities tailored to individuals' unique capacities;
- Educational workshops with guest speakers from CF centers across the country, addressing such issues as transplant, GI challenges, hemoptysis, nutrition, and sinus disease;
- Rap sessions (support groups) to support positive mental health;
- Dedicated time to connect, network, and socialize with others.

Attendees report that the retreat offers new information about CF therapies and treatments, 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 are able to invite adult family members and supportive peers.

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

CF Summer Retreat is generously sponsored by Gilead Sciences, Vertex Pharmaceuticals, AbbVie, and a designated gift from Devin Wakefield.

remain unaddressed and are very enigmatic. The Arg933 position is significantly far away from the two most known mutations, F508del and G551D, which could suggest a significantly different type of defect of CFTR. Recent breakthroughs in a technique called cryo-electron microscopy (cryo-EM) have allowed researchers to examine the atomic structure of small quantities of purified CFTR in unprecedented detail. Most notably are the remarkable cryo-EM structures determined in 2019 of CFTR bound to two different potentiator drugs, one being the FDA-approved life-saving drug Ivacaftor (VX-770).

An unexpected feature of the high-quality structures of CFTR in the presence of potentiators is that the chloride channel of CFTR is still in a closed conformation. The location of VX-770 binding on CFTR, exactly halfway through the cell membrane, is highly intriguing. The positively charged amino acid sidechain of Arg933 resides at the bilayer midpoint in an energetically costly low dielectric environment and forms a polar contact with VX-770. The Arg933/VX-770 contact is theoretically enhanced by the low dielectric and would seem to be a dominant theme of potentiator binding and function, if not an outright requirement. We explored this hypothesis by examining several mutations of Arg933. All nine mutations tested formed “band C” and several were “super-folders” with respect to wild type CFTR “band C” levels. Loss of the positive charge significantly reduced basal activity of the CFTR channel in patch clamp studies. An increase in inward rectification was also observed for several mutants. VX-770 largely stimulated channel activity of R933 mutants that showed low basal activity. CFTR-R933Y and CFTR-R933A produced clearly recognizable particles by negative stain electron microscopy (NSEM) and paves the way for high-resolution structure determination of R933 mutations to determine the role of Arg933 itself in chloride conductance and the mode of potentiator binding in the absence of the positive charge at position 933.



A Breath of Fresh Air A Gala to Support CF Research, Education, Support & Advocacy Programs

Save the date! CFRI’s Breath of Fresh Air Gala will take place on Saturday, October 14, 2023 both virtually and in person at the elegant Hillsborough Racquet Club (Hillsborough, California). Join us for this hybrid event to celebrate our strong community and exciting research progress! Those of you celebrating with us in person will be treated to delicious food and fine wines. Online attendees will join those in-person to listen to inspiring stories of hope and resilience, bid on auction items, and honor our 2023 CF Champion, an individual dedicated to improving the lives of those with cystic fibrosis.

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.

Generously sponsored to date by Vertex Pharmaceuticals, AbbVie, Chiesi USA, and Viatrix.

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

From May 5 – 7, 2023, CFRI will host its 9th annual Embrace Mothers Retreat at Vallombrosa Retreat Center in Menlo Park, CA. Through art, yoga, writing and advocacy workshops, the retreat offers an opportunity for women who share the CF path to connect and rejuvenate.

Studies show that mothers of children with cystic fibrosis have extremely high rates of anxiety and depression. This can directly impact their children’s outlook and adherence to their medical regimen. Evaluations of Embrace participants 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.



For more information and to register, please visit our website at www.cfri.org.

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

From Oral Health to CF Vests Worldwide: CF Community Voices Has Something for Everyone

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 CF and oral health, the connection between hormones, stress and inflammation, CF in Pakistan, and a podcast about Nicholas Kelly, a dietician and freestyler with CF. 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.podbean.com. You can also watch on CFRI's YouTube channel: <https://tinyurl.com/39kfd3ws>. We look forward to sharing our community's diverse voices.

CF Community Voices is made possible through grants from Chiesi USA, Genentech, Gilead Sciences, Vertex Pharmaceuticals, and Viatrix.



CFRI Is Your Partner in Living

- **PURPLE HAIR CHALLENGE:** Challenge friends and family to color their hair purple to raise CF awareness and support CFRI.
- **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.
- **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.

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 Adults Support Group

Third Monday of Every Month
May 15 • June 19 • July 17
August 21 • September 18
October 16

CF Caregivers Support Groups

Third Tuesday of Every Month
May 16 • June 20 • July 18
August 15 • September 19
October 17

Spanish-Speaking CF Community Meetings

Second Wednesday of Every Month
May 10 • June 14 • July 12
August 9 • September 13
October 11

Support Group for Teenagers with CF

Third Wednesday of Every Month
May 17 • June 21 • July 19
August 16 • September 20

Support Group for CF Adults Post-Transplant

Fourth Wednesday of Every Month
May 24 • June 28 • July 26
August 23 • September 27
October 25

Bereavement Support Group

First Tuesday of Every Month
May 2 • June 6 • July 11 • August 1
September 5 • October 3

Go to www.cfri.org for information.
All support groups are held on Zoom.

CFRI 36th National CF Education Conference

July 28 – July 30, 2023
Grand Bay Hotel San Francisco
(Redwood City, CA) and Online
(See back page for speakers, topics,
and sponsors)

CF Summer Retreat

August 18 – August 22, 2023
Vallombrosa Retreat Center,
Menlo Park, CA

CFRI's Gala "A Breath of Fresh Air"

October 14, 2023
Hillsborough Racquet Club
(Hillsborough, CA) and Online

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



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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, 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 36th National Cystic Fibrosis Education Conference: Hope on the Horizon ~ A Hybrid Event

July 28 – July 30, 2023

Grand Bay Hotel San Francisco (Redwood City, CA) and Online

The Conference will launch on Friday, July 28, with an all-day Research Track and opening reception. Speakers on Saturday and Sunday will present on topics including phage therapy, stem cell research, mRNA therapy, and parenthood with CF. Exhibitors, the annual awards dinner and dance party make this a full weekend of community connection.

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

Join us!

In-Person Registration: \$230

includes all presentations, conference meals, award banquet, receptions, support groups.

Research Track Registration: \$100 (Friday attendance only)

includes Friday presentations, Friday lunch and reception

Virtual Registration: Free

Confirmed speakers to date include:

- Stephen Aller, PhD • Saima Aslam, MBBS • Lucy Barnes • Paul Bollyky, MD, PhD & Nina Pennetzdorfer, PhD • Amanda Bruce, PhD • Matt DeFina • Suzanne Fleiszig, PhD & Naren Kumar, PhD • Deborah Friedman, PhD • Brigitte Gomperts, MD • Steven Jonas, MD, PhD • Carlos Milla, MD • Susanna McColley, MD • Kenichi Okuda, MD, PhD • Anna Payne • Deepika Polineni, MD, MPH • Alanah Rosenbloom, MSW • Carl Robinson • Zachary Sellers, MD, PhD • Ahmet Uluer, DO, MPH • Sriram Vaidyanathan, PhD

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

Generously sponsored to date by: **Viatrix, Vertex Pharmaceuticals, Genentech, Gilead Sciences, Chiesi USA, AbbVie, and the Boomer Esiason Foundation**

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