

## My CF Life Becomes the Public Reality

By Erick Burton

If you had told me six months ago that in our shared dystopian future I'd spend my days on Zoom while working from home, that I'd make wearing a mask in public fashionable and avoiding handshakes look thoughtful, or that I'd be running a health risk assessment on everything and everyone, I would have pointed out that you'd described my standard Tuesday (and everyday life) perfectly.

What a strange feeling it is to be surrounded by people forced to track and respond to the threats that have become background noise to me. What a strange mix of empathy and entertainment it is to hear friends and family complain about dry and overwashed hands, or watch them learn about how often they touch their faces, or take the mental journey from "masks don't work," to "everyone must wear masks or we'll all die."

I stopped social contact with the world shortly after the first case of COVID-19 was identified as "community spread" here in the San Francisco Bay Area. From the



Erick Burton and his wife Ashley Hartoch.

beginning, it was clear that the potential severity of the outbreak was being downplayed. Severely limited test availability and information were keeping confirmed numbers low and people calm. It did not keep me calm. I filled the vacuum of information with translated Chinese studies along with information from epidemiologists and scientists. The disease was here and

on the loose, airborne(ish), and had been spread for who knows how long by people with no symptoms.

For me, daily anxiety and fear could be measured by the difference between the data and the public response to the crisis. In late February, my wife, who works at a

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## Physiological Patient-Derived Airway Culture Models for Testing Cystic Fibrosis Therapeutics

By Martina Gentsch, PhD, Marsico Lung Institute/Cystic Fibrosis Research Center, University of North Carolina at Chapel Hill, NC

Cystic fibrosis (CF) is caused by mutations in the CFTR gene and results in inflamed and infected airways with mucus obstructions. Rescue of F508del CFTR, the most common mutation in CF, is now feasible with the recently approved CFTR modulator treatment, Trikafta. The remaining 10% of CF patients with rare CFTR mutations may also respond to Trikafta or other novel treat-

ments. However, direct correlation of in vitro laboratory data with clinical responses has not been fully established for these individuals, and they therefore remain without CFTR modulator therapies.

To optimize use of recently developed CFTR modulators for patients with rare CFTR

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## Letter from the Executive Director

Dear Friends,

I hope that this finds you and yours well. It is difficult to comprehend how radically our lives have changed since the beginning of the year, when COVID-19 was unknown to the vast majority of us. For our community, the risks associated with this virus – particularly for those post-transplant – are extreme. We are fortunate that our community's infection-control practices, which are second nature to most of us, have served us in good stead, as our rates of COVID-19 infection appear to remain lower than the general population. We have much to teach the world!

The coronavirus has spurred CFRI's activities into overdrive. Much of our programming was already virtual and continues without change. We have now transformed several key events to a virtual format, including our Mothers' Retreat, CF Adult Retreat, and National Conference. We are focused on responding to our community's needs for support and information during the pandemic, and have offered weekly Q & A podcasts on CF and COVID-19, as well as weekly support groups. Our website and Facebook pages have been updated to offer information and resources. We are forever your partner in living.

We accomplish so much thanks to our generous and compassionate organizational partners, sponsors, and community members. Our research, advocacy, education, and support services are dependent upon the generosity of our community. COVID-19 will have a dramatic impact upon CFRI's revenues this year, and I thank you in advance for your continued support.

With or without the pandemic, our loved ones still struggle to breathe, and CF remains a harsh and capricious disease. Many members of our community are still waiting for transformative therapies. Each one of us plays a part in the search for a cure.

None of us would choose this path for ourselves or our loved ones, but it is a relief to know that along with the extreme challenges, there is also camaraderie, friendship, joy, and hope. We are a strong community.

Warmly,



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



## News from the Board

Dear CFRI Community,

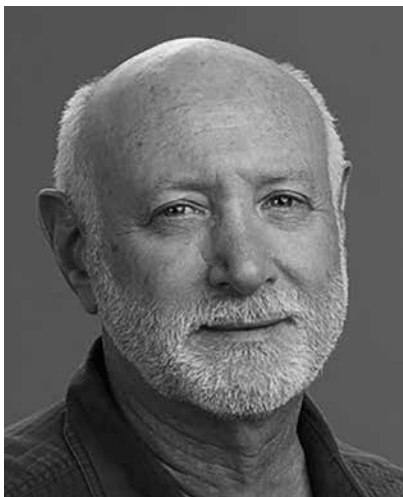
I hope that you are safe and well. As President of CFRI's Board of Directors, I have had the honor of watching CFRI's dedicated staff and volunteers respond to the COVID-19 crisis with sleeves rolled up and a determination to serve. In creating new programs, adapting existing events, advocating on behalf of those with CF, and maintaining existing services, CFRI demonstrates its responsiveness and agility in addressing our community's needs.

We began the year in excellent fiscal shape, but the impacts of COVID-19 will be significant. Difficult decisions will need to be made to maintain our level of service delivery. We cannot accomplish our work without your commitment and partnership. Whether through the Mothers' Day campaign, the Jessica Fredrick Memorial CF Research Challenge Fund, car donations, or our new Purple Hair Challenge, your participation is vital.



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# Cystic Fibrosis and COVID-19: A Q & A with Richard Moss, MD



Richard Moss, MD

Since the outbreak of the pandemic, the national cystic fibrosis community has sought information specific to CF and COVID-19. Richard Moss, MD, former Director of the Cystic Fibrosis Center at Stanford, Professor Emeritus at Stanford, and a member of CFRI's Board of Directors, has been responding to the community's questions in weekly recorded "Q & A" sessions, which can be watched on CFRI's

YouTube and Podbean channels. The following is a sampling of these questions. *Note: the following information is current as of May 4, 2020, and should not take the place of medical advice, diagnosis or treatment provided by your medical caregiver.*

## Q: Is there information on the number of CF and COVID-19 cases in the United States?

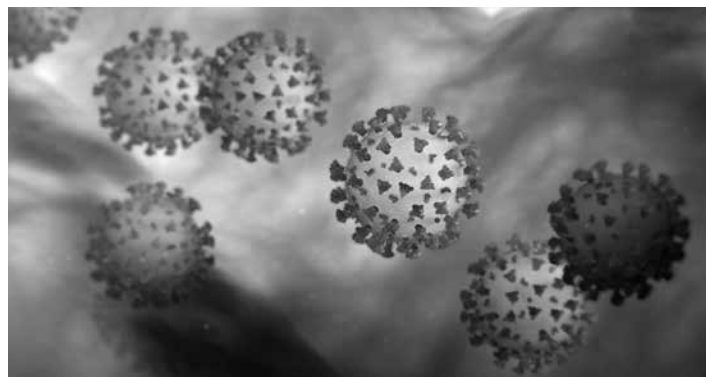
Reports from the CFF became available in mid-April; it is important to note that the foundation relies on data submissions to produce reportable information. As of April 29, there were 25 reported cases (includes 2 pediatric and 5 post-transplant), with one fatality.

## Q: Has the disease expression been more extreme for those living with CF compared to the general population?

The numbers are still small, but recent data from the Journal of Cystic Fibrosis, with input from eight countries, found that of 40 CF patients with COVID-19, 70% had recovered at the time of the report. Of the 40 cases, 11 were post-lung transplant patients. Thirteen patients needed oxygen; only 1 required mechanical ventilation. The median age was 33 years, 38% had CFRD and 70% had a chronic bacterial pulmonary infection. One was pregnant and recovered, delivering a healthy baby. Initial reports imply that the clinical course of COVID-19 in CF appears similar to the general population, with the outcomes of these first identified cases better than initially predicted. Efforts continue to obtain more information about the nature of symptoms, severity of baseline CF disease, treatments utilized, and how patients have responded.

## Q: Can you report on research and development for a COVID-19 vaccine?

Science is moving at an unprecedented speed. There are at least 86 vaccines being developed, including DNA vaccines, RNA vaccines, live attenuated and inactivated vaccines, non-replicating viral vector vaccines, and a subgroup called protein subunit vaccines, where specific parts of the virus are produced using biotechnology. At least 6 vaccines have entered clinical trials in various countries including the US, China, UK and Germany. There is still a lot we



don't know about this virus, including whether vaccine-induced antibody responses will be protective, and if so, for how long. While antibodies are one arm of our defense mechanism, we do also have a T cell-based system that may be equally important, and there's more to learn on that.

## Q: Have many of us unknowingly had COVID-19? Is a blood test the only way to know if we have antibodies?

A blood test is the only way of confirming exposure to the virus and determining if an individual has had an antibody response to the COVID-19 SARS-CoV-2 virus. Research at Stanford and USC found that 3% to 5% of the population studied showed evidence of having been exposed, meaning that 95% to 97% has not yet been exposed. About 50% to 60% of a population must carry antibodies to have so-called "herd immunity," and such studies indicate just how vulnerable we still are without widespread testing and continuous physical distancing measures in place.

## Q: What can you share about delayed clinical trials due to COVID-19?

CF research has been set back as virtually all clinical trials have been suspended and many labs closed unless studying COVID-19, and we are seeing loss of funding, interrupted timelines, and personnel changes. As we begin to recover, it will remain difficult to re-initiate trials as many biotech and pharmaceutical companies have turned their attention toward addressing the pandemic.

## Q: What should the CF community focus on to protect ourselves and our loved ones?

Fortunately the CF community is well versed in infection control practices. The COVID-19 virus attaches to many human cell types, is highly contagious and attacks multiple body systems. Fortunately, its lipid encasement makes it highly susceptible to destruction by best practice hand washing with soap and hot water, and the use of detergents to keep personal spaces clean and disinfected. Physical distancing for the general public and especially for all those with CF will need to continue, including the use of personal masks to prevent community spread. An indoor environment poses more risks than an outdoor environment. The likelihood of infection due to aerosolized particles largely depends on the concentration and viability of particles; the closer you are to a source of infection and the longer you are in contact with that person, the higher your risk of contracting COVID-19.

CFRI's CF Community Voices Podcast Series is sponsored by Vertex Pharmaceuticals, Gilead Sciences, Chiesi USA, and Genentech.

## My CF Life Becomes the Public Reality

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a hospital, was told by her employer in a “calm down conference” that there was nothing to fear from people who were asymptomatic. That was it for us; without credible safety information from her hospital, it was time to act. The fight to get her “work from home” rights was fraught with challenges because we were on the leading edge of the wave. The Stanford Adult CF Clinic came through for us with a letter and we were able to secure her right to work from home in order to keep us safe. Closing that last open loop of exposure was a profound relief.

I operate a small business without the capital necessary to weather six months to a year with minimal income. Our sales dropped to zero overnight. With no real visibility of what aid or resolution might

look like, I made the decision to shutter the company and get my employees as far to the front of the unemployment queue as I could manage. I’m hopeful we’ll be able to re-open this year. That decision has been made countless times across the country and I’ll be forever grateful to those who have kept the lights on for the rest of us (looking at you Stanford CF Research).

For me, managing the stress and anxiety of this unprecedented time has come down to a few habits that a life with CF has reinforced. Identify as many threats and benefits as I can, make a plan for each, and execute them when the time comes. Keeping the conversation this simple makes the highwire act manageable. We will return to society when the consequences of



Erick, Ashley and friends rocking masks even before it was cool. (Pre-COVID-19)

becoming ill change. If it becomes a choice between financial hardship and safety, we’re choosing safety. We will not lose focus on the importance of disciplined living in order to live well later. In the meantime, I’ll concentrate on exercise, connecting with my tribe, and losing the ‘Trikafta 15’.

## Physiological Patient-Derived Airway Culture Models for Testing Cystic Fibrosis Therapeutics

Continued from Cover

mutations, we utilized precision medicine strategies to examine CFTR rescue in patient-derived nasal and bronchial epithelial cells. Specifically, we evaluated CFTR function, protein expression, and maturation by analyzing CFTR protein levels, processing, mRNA quantities, protein-turnover, electrophysiological responses by short-circuit current, CFTR single-channel characteristics in lipid bilayers, and volume changes of spherical cultures in response to currently available modulator compounds. Using these methods, our studies revealed insights into defects and rescue approaches for many rare CFTR mutations.

In addition, we are studying the impact of the in vivo environment for CF disease prognosis, and applying this knowledge toward

improving physiological tissue-specific bioassays for therapeutic testing. We developed advanced models that incorporated the inflamed CF airway environment and observed that CF airway inflammation (induced by application of fluid from CF lungs) substantially augmented rescue of CFTR by various therapeutics. Our findings demonstrate the feasibility of in vitro evaluation of CFTR modulators utilizing personalized medicine models combining patient-derived cultures with an inflammatory stimulus acquired from the same patient. Furthermore, mass spectrometry data on pharmacokinetics and pharmacodynamics of CFTR therapeutics were collected to reveal information for optimizing drug dosage.

These biochemical and functional studies elucidated the effects of CFTR-targeting compounds on CFTR processing and function at a cellular level, with the goal of

providing predictions of clinical efficacy based on comparison of in vitro responses to in vivo outcome measures. We have demonstrated the utility of 2D planar and 3D spherical airway cultures as an assay platform to characterize rare CFTR mutations to guide therapy optimization, which illustrates the power of ex vivo and in vitro biochemical, physiological, and molecular techniques to quantitate CFTR rescue to support diagnosis and treatment.

Our studies improve the accuracy of in vitro models for CF drug development, pre-clinical testing, and evaluation of personalized therapies. This project has immediate translational potential in providing relevant models for accurate elucidation of mechanisms of CF pathophysiology and prediction of drug responses, thereby optimizing treatments that will improve the quality of life for all CF patients.

## News from the Board

Continued from page 2

The funding of cutting-edge research remains key. CFRI’s Board of Directors recently approved funding for inspired researchers at UC San Francisco and Michigan State through our Elizabeth Nash Memorial Fellowship and New Horizons Programs. In her cover article, CFRI-funded researcher Martina Gentzsch, PhD, provides an excellent overview of her work. As a member of CFRI’s community, you are a part of this innovative research.

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 | Board President

# COVID-19 and Medical Rationing: Impacts on the CF Community

By Rick Moss, MD, Ahmet Uluer, DO, MPH, and Siri Vaeth, MSW

In anticipation of healthcare staffing and resource shortages resulting from the COVID-19 pandemic, there has been much discussion about potential guidelines for determining which patients should be offered intensive treatment, including rationing on the basis of pre-existing conditions. People with chronic medical conditions and disabilities are concerned that they may be inappropriately denied access to lifesaving medical care due to disease or severity categorizations rather than carefully individualized considerations. Indeed, numerous state Crisis Standard of Care plans list cystic fibrosis with an FEV<sub>1</sub> less than 25 or 30% predicted as an example of end-stage organ failure precluding consideration for hospital admission or ventilator allocation.

These proposals raise serious concerns for people with cystic fibrosis. Currently, scant data exists on how COVID-19 manifests in patients with CF. We anticipate that, like people with other underlying respiratory conditions, people with CF are at increased risk of critical illness if infected with COVID-19. Increased rates of diabetes in adolescents and adults with CF also contribute to risk of complications. However, evidence from China suggests that the majority of people

who contract COVID-19 survive, even those who have underlying respiratory conditions or diabetes.

The restrictions based upon predicted FEV<sub>1</sub> found in many state crisis plans trace back to exclusion criteria proposed in a model protocol by researchers as part of the Ontario Health Plan for an Influenza Pandemic. The justification provided for these criteria is that they align with 1998 transplant eligibility guidelines, which “typically represent a baseline death rate [without transplantation] higher than 50% within the next 1 to 2 years.”

However, more recent data disproves this outdated prognosis. A 2017 retrospective cohort analysis of over 3,000 patients in the Cystic Fibrosis Foundation Patient Registry with FEV<sub>1</sub> < 30% predicted found that median transplant-free survival was 6.6 years after reaching this benchmark. While the CF Foundation continues to recommend all patients with a stable FEV<sub>1</sub> under 30% predicted be referred for transplant evaluation, they also note that prolonged survival is possible for patients in this category. The long-term impact of new CFTR modulators will not be known for some time; however,

short term data reverses previous clinical assumptions for those with FEV<sub>1</sub> <40%. The rationale that such patients should be excluded from consideration for mechanical ventilation on the basis of poor survival is clinically unjustifiable.

Any judgment of futility must be based on a more nuanced assessment of individual clinical history, not simply a diagnosis. Should rationing become a necessity, there are suggested frameworks that utilize measurement scales – not unlike those utilized for solid organ transplantation – to integrate multiple criteria into a single tool for care teams to utilize in the face of difficult allocation decisions. Patients with CF should have the same opportunity to access care as any patient without an underlying condition.

CFRI has been actively involved in advocating for changes in targeted state crisis plans. State policymakers, hospital systems and other entities considering how to allocate scarce medical resources should revise their allocation criteria to remove this inaccurate restriction, lest they inadvertently deny access to treatment for a population for which it may well be justified.

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

Due to the COVID-19 pandemic, our Embrace Retreat, previously scheduled at Vallombrosa Retreat Center in Menlo Park, was held virtually this year. While there is no substitute for in-person interaction, the virtual version was a success, with 23 mothers participating from across 5 time zones – from Hawaii to Vermont. In advance of the full day retreat, online writing and art workshops were offered, allowing participating mothers to reconnect and get acquainted. On the day of the event, attendees participated in presentations, therapeutic art and writing workshops, yoga, and an overview of additional resources offered by CFRI throughout the year to provide lasting support.

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, and their family’s well-being. Evaluations of Embrace participants show that the retreat is extremely effective in lowering depression and anxiety. Participating in a variety of workshops and activities while connecting with others who



share the CF path helps mothers to build upon their inner strength and resilience to be better prepared for the ongoing challenges presented by this disease.

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

# 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 while you shelter in place! 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.

**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://www.classy.org/campaign/purple-hair-challenge/c284589>

## Awareness Building with Your Electeds:

Due to COVID-19, many in-person CF awareness events were cancelled. To ensure that our community's voice is heard, and that our elected representatives remain aware of the challenges we still face, we have created an easy way to send emails to both your state and Federal representatives. Go to [www.cfri.org](http://www.cfri.org) and click on the "Raising Awareness with Our Elected Representatives" link. By entering your zip code, your message will automatically be sent to all those who represent you.

## Virtual Live and Love National Yoga Workshop:

CFRI is hosting a live donation-based yoga workshop on Sunday, May 31st, at 10:00 am PT, to bring us all together in mindfulness, breath, and movement to honor CF awareness month and our community. To sign up, go to our Events page at [www.cfri.org](http://www.cfri.org).



## Tributes

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

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



[www.donatelife.net](http://www.donatelife.net)

# In Honor of

September 1, 2019 — March 15, 2020

Mr. and Mrs. Gordon  
Adelman  
Sandra Aguirre  
Claire Alexander  
Alyssa  
Sadie Anderson  
Kyle Baker  
Bridget Barnes  
Lucy Larkin Barnes  
and her family  
Darrell Batchelder  
The Baugh Family  
Jamie Beasley-Killinger  
Maggie-Faye Bendz  
Alison Best  
Francine Bion  
Micki Boswell  
Lacy Bradley  
Lucas Buchanan  
Brian Burks  
The Burks Family  
Isabel Stenzel Byrnes  
Traci Carwana  
Children's Hospital Oakland  
John Christie  
Shaun Collins  
Lauren Colonna Cooper  
Joanna Conroy  
Cameron Cornell  
Jordan Cote  
Timothy Crose  
Barbara and Jim Curry  
Bradley Daniel  
Tracy Del Real

Edna and Chuck DeVore  
Gordon DeVore  
Mackenzie Dondanville  
Luke Dorman  
Ann Du Frane  
Dylan Dunn  
Tess Dunn  
Savannah Durand  
Ed  
Timothy Estabrook  
Janelle and Andre Estournes  
Faith Ettlich  
Tommy Evans  
Jarrod Fischer  
Victoria and Oscar  
Flamenco  
Kathleen Flynn  
Jessica Franklin  
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Gaynell Gleason  
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Andy Gordon  
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Gianna Gutierrez-Serrato  
Natalie Hanson  
The Hardy Family  
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Shae Kish-Walters  
Kristin Konvolinka  
Santosh Krishnan  
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Dan and Doug Lagasse  
Stacey Lawn  
Theresa Le Berthon  
Dylan Leaphart  
Maureen and Nick Lenssen  
Megan Lepore  
Owen Litzenberger  
Michael Livingston  
Joseph Arthur Lopez  
Josh Loux  
Emily Fredrick Lucas  
Eric Marten  
David Martin  
Tessa McCarthy  
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Luanne McKinnon

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Sara Modlin and Ian Tucker  
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Angela Morey  
Ellen Neidle  
Jessica Nett  
Lindsey Nijmeh  
Tristan O'Neill  
Michael Orlen  
Charlotte and Joseph Paliani  
Bill Patterson  
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Robyn Primack  
Justin Raines  
Michael Reuscher  
The Reynolds Family  
Joe Rigge  
Mike Roanhaus and Family  
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Rob Robinson Family  
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# In Memory of

September 1, 2019 — March 15, 2020

Briana About  
Chelsa About  
Sylvia Allen  
Gianna Altano  
David and Jodi Armknecht  
Rose Balsley  
Sally Bariloti  
Cara Baysinger  
Anne Beltrame  
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Sammy Cassidy  
Sonya Chartrand  
Cassandra Cochran  
Howard Cochran  
Irene Colonna  
Caroline Daly  
James and Norma Delehanty  
Neva L. DeVore  
Anthony Di Profio  
Vicki Dippner-Robertson

Maxine Eggerth  
Jennifer Eisner  
John Fambrini  
Rosemary Farac  
David Fisher Sr.  
Marvin Fox  
Jessica Fredrick  
Laura Gale  
Aaron Gallagher  
Theresa, Rita-Jean  
and Cathy Gaspari  
Wallace Erby George  
Diana Goodman  
Ernie Gotti  
Merwin Gunter  
Lise Hardin  
David Hardy  
Katie Hauger  
Nicholas Hollis  
John Holmes  
Merrilyn Holmes  
Leslie Hotson  
Robert and James Houston  
Robert Huntzinger  
Sean Hyland  
Orzella Jemas  
Brian Jensen  
Edward Jensen  
Edwin Johnson  
Karen Johnson  
Melody Johnson  
Gary Judd  
Kathy and Peter Judge

Linda Junge  
Blake, Stan and Shirley Kelley  
Jeff Kennedy  
Peggy and Janet Kessel  
Kitty Kious  
Lori Kipp  
Bridget and John Klein  
Eleanor Kolchin  
Jason Konkel  
Jeffery Kreth  
Jane Ellen Kulik  
Mary Kulik  
Diana Lamark  
H. Lane  
Grant Lindee  
Alan Linder  
Dawn Longero  
Alyson Lowery  
Robert Mackey  
Krista Malone  
Zoe Mann  
Richard Marias  
Lucille Rose Cynthia Marsh  
Wayne Martinelli  
Catharine Martinet  
Nahara Mau  
David McAfee  
Bill McDow  
Stacy Melle  
Linda Meyer  
Jessica Miller  
Madeline Montelatici  
Clyde and Ross Mosier

Maurice Moyle  
Lynn Moyse  
Kimberly Myers  
Elizabeth Nash  
Kate Nelson  
Kim, Scott and Jody Nelson  
Leroy Nelson  
Elisabeth Nichols  
Michele D. Olson  
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John Sentman  
Declan Sheedy  
Steven L. Shepherd

Ron Shirley  
Tammy Smerber  
Anabel Mariko Stenzel  
Kim Stewart  
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# CFRI Needs Your Support – Now More Than Ever!

## 2020 Mothers' Day Fundraiser Brings Awareness and Hope!

Mother's Day is just the beginning! CFRI's 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 nearly \$110,000 to support CFRI's vital services to the CF community.

In light of the significant impacts of COVID-19, this will be a challenging year for CFRI. Those with cystic fibrosis are extremely vulnerable to the ravages of COVID-19, and the fear wrought by the virus

is extreme. Your participation will have a meaningful impact on raising needed awareness and revenues.

Before our world changed so radically, we were celebrating recent therapeutic advancements that provide better health to many living with CF. Sadly, the much-heralded new CFTR modulator therapy cannot be used by thousands of people with CF.

This year's cards feature beautiful artwork created by five-year-old Natalie Puzia, an adorable girl with a rare mutation that is not responsive to the new therapies.

No matter how you would like to send your Mothers' Day invitations – in the mail, online or through Facebook - you can call or email Mary at 650.665.7559 or [cfri@cfri.org](mailto:cfri@cfri.org) to assist you, or go to <http://cfri.org/mothers-day-celebration/>.



## CFRI's Response to COVID-19: Informing, Supporting and Advocating for the CF Community

The devastating impact of COVID-19 is being experienced around the globe. For those with cystic fibrosis, this pandemic has triggered deep concern on many levels. While there is an endless stream of COVID-19-related news, there is very little specific to cystic fibrosis. CFRI is committed to providing our CF community vital information, resources and support, while advocating on its behalf at the state and federal level.

### Information:

- **Website:** CFRI's website is regularly updated to provide current COVID-19-related information and resources for the community.
- **COVID-19 Podcasts/YouTube Playlist:** Since our virtual Town Hall meeting on COVID-19, we have released weekly Q & A sessions with Dr. Rick Moss of Stanford, who answers questions submitted by CF community members. These podcasts are available on CFRI's YouTube channel.
- **eNewsletter:** Our weekly eNewsletter contains a wealth of information on COVID-19 developments. Please email [cfri@cfri.org](mailto:cfri@cfri.org) if you are not subscribed.

### Support:

- **Weekly COVID-19 and CF Support Group:** Since March, CFRI has hosted an online discussion/support group for CF community members to share experiences and address COVID-19-related anxiety. The group is moderated by Yelizaveta Sher, MD, psychiatrist at Stanford's Adult CF Center.
- **Ongoing Support Groups, Counseling Support and Wellness classes:** Now more than ever, CFRI's CF Quality of Life and Wellness programs are playing a key role in supporting the mental health of our community.

### Advocacy:

CFRI has advocated on its own and in partnership with other health-related organizations at the state and federal level on issues resulting from COVID-19. These include:

- **Medical Rationing:** CFRI advocated to US Department of Health and Human Services Secretary Alex Azar, state health departments, and state governors to urge the removal of discriminatory language from state emergency plans

that contain outdated information that excludes or deprioritizes those with advanced lung disease from receiving ventilators should there be a shortage – including those with FEV<sub>1</sub> of less than 30%.

- **Access to Telemedicine:** Along with our rare disease partners, CFRI urged governors whose state licensure requirements did not permit telemedicine across state lines to adjust these requirements during the COVID-19 crisis.
- **Expansion of Paid Medical Leave:** CFRI, along with 150 other organizations, co-signed a letter generated by the CFF asking Congress to expand paid family medical leave for people with chronic conditions and their care givers.
- **Early and Expanded Refills:** CFRI targeted state insurance commissioners to advocate for early and extended refills of prescription medications so as to allow individuals with CF to safely shelter in place.

CFRI will continue to keep our community informed, supported and engaged as we cope with the challenges of COVID-19.



# CFRI Research Awards Program Supports Promising Cystic Fibrosis Research Nationwide

## Through New Horizons Research Campaign and Elizabeth Nash Memorial Fellowship

### New Horizons

Through the New Horizons grants, CFRI funds research of the highest scientific quality in academic and hospital institutions. The program provides a \$70,000 award per year for two years to Principal Investigators. Funded projects are highly relevant to CF and are original, probing and/or pioneering a new approach to a therapy or cure. The NH awards also provide seed funding for promising new lines of basic and clinical CF research that show prospects of future funding by other sources, such as the National Institutes of Health (NIH).

### Current New Horizons Researchers:

- Martina Gentzsch, PhD: Physiological Models of Cystic Fibrosis Airway Cultures that Mimic the In Vivo Environment for Therapeutic Testing; *Principal Investigator, University of North Carolina at Chapel Hill*
- Lucas Hoffman, MD, PhD: Bacterial Community-Level Susceptibility Testing for CF Lung Infections; *Principal Investigator, University of Washington, Seattle and Seattle Children's Hospital*
- Elizabeth Kramer, MD, PhD: The Role of TGF in Driving Airway Hyperresponsiveness and Smooth Muscle Dysfunction in Cystic Fibrosis; *Principal Investigator, Cincinnati Children's Hospital Medical Center*
- Kenichi Okuda, MD: Regional Regulation of CFTR and Ionocyte Expression in Airways; *Principal Investigator, University of North Carolina Chapel Hill*
- Forest Rohwer, PhD: Development of Tailocins Against Microbial Infections in Cystic Fibrosis Lungs; *Principal Investigator, San Diego State University*

### Incoming 2020 Researcher

- Xiaopeng Li, PhD: Targeting V-type ATPase in Human Small Airways for CF Lung Disease Treatment; *Principal Investigator, Michigan State University*

### Elizabeth Nash Memorial Fellowship

The Elizabeth Nash Memorial Fellowship (ENMF) provides annual funding to Postdoctoral Fellows engaged in original, probing and/or pioneering CF-related research at academic and hospital institutions nationwide and encourages collaborative research and communication between the institutions and Principal Investigators. The fellowship is named in memory of geneticist Elizabeth Nash, who had CF and served as CFRI's RAC Chairperson. Up to \$60,000 per year for two years is awarded to a research institution to support a principal investigator, who in turn appoints a postdoctoral research fellow.

### Current Elizabeth Nash Memorial Fellowship Researchers include:

- Ana Cobian, PhD: Bacteriophage-Mediated Spread of Virulence Factors in Cystic Fibrosis Microbiomes; *Postdoctoral Fellow, San Diego State University (Forest Rohwer, PhD, Principal Investigator)*
- Kehoon Lee, PhD: A Multi-'Omic Approach to Evaluate Concurrent Sinus and Pulmonary Disease in Cystic Fibrosis; *Postdoctoral Fellow, Northern Arizona University (Emily Cope, PhD, Principal Investigator)*

### Incoming 2020 Researcher

- Kelly Tuveson, DO: In Vitro CFTR Function in Children with CFTR Related Metabolic Syndrome; *Postdoctoral Fellow, University of California San Francisco (Ngoc Ly, MD, MPH, Principal Investigator)*

## 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. Last year, members of our circle contributed over \$90,000 so as to match – dollar for dollar – donations from individuals committed to CF research. Together, these donations were used for our New Horizon and Elizabeth Nash Memorial Fellowship CF research awards.

Please join this inspiring group! Become a member of the 2020 Jessica Fredrick Memorial CF Research Challenge Circle by making a minimum gift of \$2,500. You will

help inspire others to make the dream of a CF cure a reality.

Our Circle is named in memory of Jessica Fredrick, pictured right, who 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. There is still no cure. We need your help to improve and save the lives of our loved ones.

Please give today. 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.



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

Prior to the stress of COVID-19, those impacted by cystic fibrosis (CF) already demonstrated higher rates of depression and anxiety than found in the general population. With its unpredictability, daily treatment burden, and diverse symptoms, cystic fibrosis is a challenging disease for those diagnosed, as well as for those who love them. Making the issue more complicated, studies show that depression can negatively impact adherence to one's medical regimen. In response, 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.
- **Caregivers Support Groups:** Two groups are offered – one for parents of children with CF, and another for parents/spouses/partners of adults with CF – and facilitated by CF social workers Meg Dvorak, LCSW, and Kate Yablonsky, LCSW. The groups are held via Zoom, and caregivers participate from across the country.
- **CF Adult Support Groups:** Adults with CF are invited to this support group, held the third Monday of every month and facilitated by CF social workers Meg Dvorak, LCSW, and Kate Yablonsky, LCSW. The groups are held via Zoom, and caregivers participate from across the country.
- **“Living Mindfully with CF” Online Classes:** Drawing upon Mindfulness Based Stress Reduction techniques and focusing these practices to living with CF, this class helps to reduce anxiety and depression and is offered to individuals with CF and their family members. Taught by Julie Desch, MD, who herself lives with CF.
- **CF Bereavement Group:** For those who have lost a loved one to CF, this online group will include sharing and discussion, journaling/writing, goal setting, grief education, and self-care strategies, and will be led by Isabel Stenzel Byrnes, LCSW, MPH, bereavement social worker who herself lives with CF.

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](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, Horizon Pharma, Translate Bio, and contributions through CFRI's CF Quality of Life Program, a Living Legacy of Peter and Kathy Judge.*

## 2020 Virtual CF Summer Retreat: A Place of Community for Those with CF July 2020

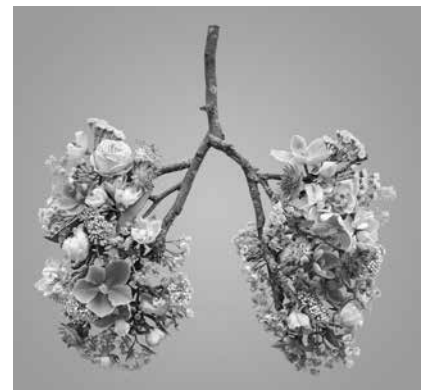
CFRI's CF Summer Retreat provides a safe and welcoming environment for adults with cystic fibrosis, providing information and social support to those who are increasingly isolated due to cross-infection protocols. **This year the retreat will be fully virtual**, and will provide health-related and psychosocial support activities including exercise, presentations on CF therapies by national experts, support groups, and time to socialize and connect. The retreat is open to adults with CF as well as their adult family members and supportive peers. **Join us!** For more information, go to [www.cfri.org](http://www.cfri.org) or call Mary Convento at 650.665.7559. *CF Summer Retreat is generously sponsored by Gilead Sciences and AbbVie, with additional support from Genentech.*

## From a COVID-19 Playlist to CF Mental Health: CF Community Voices Has Something for Everyone

By the community and for the community, CFRI's CF Community Voices was created to share information and insights about a wide variety of topics. Recent releases include the weekly COVID-19 Q & A with Dr. Rick Moss, and episodes on CF and Bone Health, GI Issues and Mental Health. New episodes are released monthly, and can be downloaded on CFRI's podhosting site: [cfri.podbean.com](http://cfri.podbean.com). You can also watch on CFRI's YouTube channel. We look forward to sharing our community's diverse voices. *Generously sponsored by Vertex Pharmaceuticals, Chiesi USA, Gilead Sciences, and Genentech.*

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

CFRI's Breath of Fresh Air Gala was scheduled to be held Saturday, October 17, 2020 at the beautiful University Club of Palo Alto, in Palo Alto, California. Due to safety measures related to COVID-19, we will likely offer an alternative date or virtual event. Should we reschedule to a later date, you will enjoy gourmet delicacies and fabulous entertainment while honoring our 2020 CF Champion and celebrating progress in CF research and therapies. Should we move to a fully virtual event, we promise it will be engaging and exciting! We look forward to bringing our community together to raise needed funds for CFRI's research, education, advocacy, and support programs to improve the lives of those with CF.



**Cost: TBD.** Sponsorship packages are available. For more information go to [www.cfri.org](http://www.cfri.org), or call 650.665.7586. *Generously sponsored to date by Vertex Pharmaceuticals, AbbVie, Chiesi USA, and Genentech.*

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

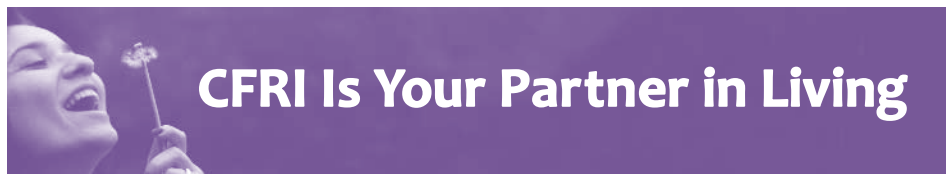
CFRI's wellness programs were developed in recognition of the positive impact of movement and exercise upon one's physical and mental health. These online programs are free, fun and interactive, and are open to those with CF, as well as their parents, spouses, partners and siblings nationwide. Working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



In 2020, CFRI is offering yoga, physical therapy, and strength-building classes. By exercising together, our community builds emotional and physical resilience, while forging new connections and friendships.

For the current schedule, go to the events page at [cfri.org](http://cfri.org).

CFRI's CF Wellness Initiative is supported to date through a grant from Vertex Pharmaceuticals



## 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, yoga, Pictionary challenge – no idea is too big or too small. Create an event, and we'll help you make it happen.
- **FACEBOOK:** Many community members create fundraisers for CFRI by donating their birthdays on Facebook. Go to <https://www.facebook.com/cfri.org/>, scroll down to Fundraisers, and click on Create!
- **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. To participate, go to our website or contact Stacie Reveles (see below).
- **TRIBUTES:** "In Honor Of" and "In Memory Of" – Recognize a loved one with your choice of gift. CFRI will promptly send an acknowledgement letter to your designee.
- **STOCK DONATIONS TO CFRI:** Donating appreciated stock avoids capital gains taxes incurred had the stock been sold. You're also entitled to an income tax charitable deduction for the stock gift date's fair market value.
- **PLANNED GIVING:** Offers benefits that can 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 Programs and Advocacy Associate: 650.665.7586 or [sreveles@cfri.org](mailto:sreveles@cfri.org).

## SAVE THE DATES!

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

### CF Caregivers Support Groups

Third Tuesday of Every Month  
 May 19 • June 16 • July 21  
 August 18 • September 15  
 October 20

Go to [www.cfri.org](http://www.cfri.org) for information.  
 Participate by Zoom or by phone – no in-person meetings until further notice  
 Sponsored by Vertex Pharmaceuticals, Horizon Pharma, Gilead Sciences, and Chiesi USA

### CF Adult Support Groups

Third Monday of Every Month  
 May 18 • June 15 • July 20  
 August 17 • September 21  
 October 19

Go to [www.cfri.org](http://www.cfri.org) for information.  
 Participate by Zoom or by phone  
 Sponsored by Vertex Pharmaceuticals, Horizon Pharma, Gilead Sciences, and Chiesi USA

### CF Summer Retreat

July, 2020

Due to the COVID-19 pandemic, the Retreat will be fully virtual  
 Sponsored by AbbVie and Gilead Sciences

### CFRI's 33rd National CF Education Conference

August 1 – 2, 2020

Due to the COVID-19 pandemic, the Conference will be fully virtual  
 (See back page for schedule and sponsors)

### CFRI's Gala "A Breath of Fresh Air"

October 17, 2020

University Club  
 Palo Alto, CA

Sponsored by Vertex Pharmaceuticals, AbbVie, Chiesi USA, and Genentech

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

*Cystic Fibrosis Research, Inc. exists to fund research, to provide education and personal support, and to spread awareness of cystic fibrosis, a life-threatening genetic disease.*

## CFRI Vision

*As we work to find a cure for cystic fibrosis, CFRI envisions informing, engaging and empowering the CF community to help all who have this challenging disease attain the highest possible quality of life.*

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

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

# CFRI's 33rd National Cystic Fibrosis Education Conference: Partners in Progress ~ An Exciting Virtual Experience August 1 – 2, 2020

By necessity, our 33rd annual conference will be offered in an exciting and engaging virtual format, offering outstanding speakers, support groups, and activities, as well as the opportunity to connect with your CF community from across the country and globe. We are taking virtual conferences to a whole new level. **Join us!**

### Speakers Include:

- James F. Chmiel, MD, MPH
- Douglas Conrad, MD
- Julie Desch, MD
- Marie Egan, MD
- Dennis Hadjiliadis, MD, MHS
- Nicole Irizarry, PT, DPT, CCS
- Richard Moss, MD
- C. Virginia O'Hayer, PhD
- Caroline Okorie, MD, MPH
- Diane Shader Smith
- Yelizaveta Sher, MD, FAACP
- Sriram Vaidyanathan, PhD

### CFRI-Funded Researchers:

- Ana Georgina Cobián Güemes, PhD
- Martina Gentzsch, PhD
- Lucas Hoffman, MD, PhD
- Elizabeth Kramer, MD, PhD
- Keehoon Lee, PhD
- Kenichi Okuda, MD, PhD
- Forest Rohwer, PhD

Generously sponsored by: **Vertex Pharmaceuticals, Genentech, Gilead Sciences, Chiesi USA, AbbVie, and Ionis Pharmaceuticals**

**Cystic Fibrosis Research, Inc.** a 501(c)(3) nonprofit organization Federal EIN# 51-0169988



### Registration is Free!

The first 100 registrants will receive a complimentary copy of *Salt in My Soul*, by Mallory Smith!

For more information, visit [www.cfri.org](http://www.cfri.org) or call 855.cfri.now.

