

## CFRI's 32nd National Cystic Fibrosis Education Conference: Speaker Abstracts

At CFRI's 32nd National CF Education Conference, *Charting the CF Course*, held July 26 to 28, 2019, nationally-renowned speakers from across the country shared their expertise and experience on a wide range of CF-related topics. Eight of these presentations were recorded and are available for viewing on CFRI's YouTube channel. The abstracts below provide a brief overview of each talk, followed by a link to the specific presentation.

### The JEM Show – Jerry & Em: Living... Breathing... Succeeding in Life with CF

Jerry Cahill & Emily Schaller — Boomer Esiason Foundation & Rock CF Foundation

While opening up about having cystic fibrosis was never something he did when he was younger, today Jerry shares his story of growing up in the dark ages of CF, being forced to retire due to the progression of his disease, and how he became an advocate for a community that is so often under-represented. Follow along as Jerry reveals how exercise has kept him healthy for 63



**Save the Date!** CFRI's 33rd National Cystic Fibrosis Education Conference will be held July 31 – August 2, 2020!

years – before and after a double lung transplant – and what drives him to be relentless in the face of all the adversity cystic fibrosis places in his way. As a CF Ambassador, Jerry also talks about the incredible work that the Boomer Esiason Foundation has done over

the past 26 years and how he has contributed to those accomplishments.

Mixing equal parts wit and humor, Emily lets the crowd sit “shotgun” as she tells her journey of living three very different decades

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## Bacterial Viruses in Cystic Fibrosis Lungs: From Agents of Bacterial Virulence to Personalized Medicine for CF — Cynthia Silveira, PhD



Cynthia Silveira, PhD

Phage therapy is a potential tool to kill antibiotic-resistant bacterial infections. Phages are viruses that infect and kill bacteria. However, phages can also maintain long-term associations with bacteria, during which phages carry genes that modify bacterial behavior, often increasing virulence and antibiotic resistance. Therefore, the efficient and safe use of phage therapy requires a better understanding of the dynamics of natural phage communities present in cystic fibrosis (CF) patients' lungs. Our lab is currently developing approaches to investigate the individualized microbiomes of CF patients with focus on phages.

The Cystic Fibrosis Rapid Response (CFRR) follows phage and bacterial infections in patients undergoing acute decline in lung function that are unresponsive to traditional antibiotic treatment. These cases are analyzed using modern microbiome tools including viromics, metagenomics, and transcriptomics at a clinically-relevant time scale. During a CFRR, samples from an exacerbating patient are processed and analyzed within two days. This study has shown that during rapid lung function loss, the phage communities change their infection strategies, killing some bacterial groups, while staying dormant in the genomes of others. During these longer-term genomic associations, we identified genes encoded by the phages that

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Fall 2019

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## Letter from the Executive Director


Dear Friends,

It is an exciting time for the cystic fibrosis community. The Food and Drug Administration's (FDA's) recent approval of the triple combination CFTR modulator therapy, Trikafta, will provide a meaningful treatment option for up to 90% of those with CF. For many, this will be their first experience with a CFTR modulator. The FDA's rapid review and approval of the therapy – in approximately 90 days – speaks to the effectiveness of the drug as well as to the power of our community in conveying our urgent need for new therapies. Through our 2018 Externally-Led Patient Focused Drug Development Meeting on CF and beyond, we shared our community's struggles and challenges with representatives of the FDA. Our voices were clearly heard.

But of course, there are many with cystic fibrosis who are ineligible to use this drug, based on their CFTR mutations. While we celebrate, we also remain focused on the 10% of those with rare mutations who still have no new options. We still have much work in front of us. And we must remember that a cure still eludes us.

As 2020 approaches, we are planning our programs and services to best meet the needs of our diverse community. Our educational programs, advocacy efforts, psychosocial support, and research awards can only thrive with your participation. Please engage with us! Your involvement is crucial if we are to remain organizationally nimble and responsive to the evolving needs of our community. I look forward to working with you!

Warm regards,



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



## News from the Board

Dear CFRI Community,

I hope this finds you well. It is an honor to serve as President of CFRI's Board of Directors. I am forever impressed by the synergy of organizational forces that result in top notch programs being offered to our CF community.

I am thrilled to announce that CFRI's effectiveness and efficiency has led to our fourth consecutive 4-star rating from Charity Navigator. This rating was received due to our demonstration of strong financial health and commitment to accountability and transparency. CFRI's clean audit has been completed, documenting another successful year for CFRI. As the enclosed Annual Report demonstrates, thanks to the generous support of our sponsors, grant funders and donors, we raised over \$1.7 million dollars.

CFRI runs a very lean operation so as to focus on growing our funding for innovative research, psychosocial support, education, and advocacy efforts. The enclosed Annual Report demonstrates the impact and reach of these key programs.

We are all partners in living. Thank you for your involvement with – and support of – CFRI and our CF community.

Peace and good health,



Bill Hult | President, CFRI Board of Directors





Emily Schaller & Jerry Cahill

decades with cystic fibrosis. Diagnosed in 1983 when she was 18 months old, Emily is now 37 and has never been more determined to outrun cystic fibrosis. Emily discusses how she re-found exercise, switched from the typical “CF Diet” to a whole-food based vegetarian diet, the importance of participating in clinical trials, and the benefits she has experienced from an epic CF breakthrough. As founder of the Rock CF Foundation, she also talks about the impact that Rock CF continues to have on the global CF community.

Watch Jerry and Emily's presentation at <https://tinyurl.com/yy29xw6j>

### The Transformation of Cystic Fibrosis Therapy

Steven Rowe, MD, MSPH  
University of Alabama at Birmingham

Cystic fibrosis is an autosomal recessive genetic disorder caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, which encodes an anion channel expressed on the epithelial surface. Through an increased understanding of CF pathobiology and the partnership of academic and pharmaceutical research

Steven Rowe, MD, MSPH



enterprises with a multinational team of care centers, CF care represents a model for the treatment of chronic, life-threatening disease. In recent decades, CF outcomes have steadily improved through multi-disciplinary and aggressive CF care

targeting nutrition, infections and comprehensive care. More recently, the care of CF has begun a transformation fed by advances directed at the underlying pathobiology of the disease. CFTR modulators, including CFTR potentiators which augment channel gating of CFTR channels localized at the cell surface, and CFTR correctors which restore F508del and other related mutations to the cell surface, have recently been approved for use and provided substantial clinical impact while fundamentally altering disease pathogenesis. The next generation of CFTR modulators are in development and promise even greater efficacy to a wide variety of mutations – including those with a single copy of F508del mutation – and other technologies in development for rare CFTR alleles. Interventions targeting abnormal mucus may overcome deficits of CFTR for difficult to treat mutations and those with established disease in which bronchiectasis itself accelerates progression. Development of these and other interventions will be facilitated by imaging technologies that visualize the CF mucus defect at its origins. In this way, the story of CF provides a seminal example of the potential for translational medicine to deliver scientific advances that have transformed a universally fatal condition by the advent of comprehensive care centers and targeted therapeutics. Future advances will continue to capitalize on personalized therapies directed at the underlying cause of the disease for all patients with CF.

Watch Dr. Rowe's presentation at <https://tinyurl.com/y55e4qb4>

### Let's Talk About Sex: Improving Sexual and Reproductive Health Care in Cystic Fibrosis

Traci Kazmerski, MD, MS  
UPMC Children's Hospital of Pittsburgh, Pittsburgh, PA

With over half the cystic fibrosis (CF) population now over the age of eighteen, CF teams must adapt to the developing needs of the adult population. Adolescents and young adults with CF view sexual and reproductive health (SRH) as a key aspect of their emerging adulthood and comprehensive care. This is not surprising as SRH decisions are often the first medical care many teens and young adults seek independently from their parents and families. A vital part of “adulting” is making personal SRH decisions.

People with CF experience a variety of general and disease-specific SRH concerns. Women may experience delayed puberty, worsening CF symptoms during their menstrual cycle, urinary incontinence, vulvo-vaginal yeast infections, sexual functioning concerns, issues around contraceptive choice, decreased fertility and complex challenges during pregnancy. For men with CF, over 97% are



Traci Kazmerski, MD, MS

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## Food and Drug Administration Approves Breakthrough Triple Combination Therapy for Cystic Fibrosis

On October 21, 2019, the U.S. Food and Drug Administration (FDA) approved Trikafta, the first triple combination CFTR modulator therapy for cystic fibrosis (CF). Trikafta is approved for individuals with CF 12 years or older, who have at least one copy of the F508 del mutation, thereby providing a therapy to nearly 90% of those with CF.

Trikafta, made by Vertex Pharmaceuticals, was reviewed and approved in only three months. Said acting FDA Commissioner Ned Sharpless, M.D., “At the FDA, we’re consistently looking for ways to help speed the development of new therapies for complex diseases, while maintaining our high standards of review.”

Two trials involving 510 patients with CF confirmed the efficacy of Trikafta. The first trial demonstrated a mean ppFEV1 increase of 13.8% from baseline compared to placebo as well as improvements in respiratory symptoms, while the second study demonstrated a reduced rate of pulmonary exacerbations and improved BMI.

infertile due to the congenital absence of the vas deferens and many may also experience delayed puberty, urinary incontinence, decreased volume of ejaculate and concerns with their sexual function.

As life expectancy improves more people with CF are considering parenthood. However, reproductive decision-making for women and men with CF is complicated. Some important considerations include the effect of pregnancy and/or parenting on health, the risk of CF in offspring, and the practical, emotional and ethical considerations of raising children while facing increased morbidity and reduced life expectancy.

Despite the fact that people with CF engage in the same types and rates of sexual activities as peers without CF, they report lower rates of SRH care utilization (including STI and pregnancy testing and cervical screening) compared to the general population. They also report that SRH is rarely discussed within the context of their CF care.

Many people with CF view their subspecialist as their de-facto primary care provider. Thus, as they may selectively seek SRH care from their CF team, building collaborative models that harness the expertise of all health care providers may help with SRH care delivery and education. The CF team should routinely address SRH and coordinate services with primary care and reproductive health specialists. Optimizing SRH care for those with CF may lead to improvements in overall transition readiness, ownership over other disease-specific aspects of health and quality of life.

Watch Dr. Kazmerski's presentation at <https://tinyurl.com/yxa29e9p>

### Motivating Behavior Change in Individuals with CF

Nadia Islam, PhD  
The Pediatric Center, Henrico, VA

One of the most challenging aspects of living with cystic fibrosis is the intensity of treatment demands and the critical importance of adherence. Healthcare providers and loved ones of individuals with CF often struggle with how to support treatment adherence and motivate behavior change. Often well-intentioned strategies of reminding, nagging, lecturing and warning have the opposite effect. Instead of increasing

adherence, these approaches can lead to tension, conflict, resentment, guilt and even hopelessness. Motivational interviewing (MI) is a promising approach to effectively improve treatment adherence, decrease conflict around treatments, improve relationships and foster a stronger sense of autonomy and confidence.

MI is an evidence-based style of communication that is collaborative, non-judgmental, patient-directed and goal oriented. MI has been successfully taught to and used by healthcare providers to improve treatment



Nadia Islam, PhD

adherence and positive health behaviors with a wide range of illnesses including substance abuse, obesity and diabetes. Research has shown MI is especially effective when the individual experiences ambivalence about changing the behavior. Unfortunately, research related to MI and treatment adherence in CF is limited and many CF healthcare providers lack training and confidence in the use of MI. Moreover, parents and loved ones of individuals with CF typically receive little to no guidance in how to effectively increase motivation and support behavior change, let alone education on helpful MI skills.

This presentation introduced basic MI skills that healthcare providers, parents and loved ones of individuals with CF can use to increase motivation and support positive behavior changes. The presentation included considerations for how MI can be adapted depending on the developmental level of the individual with CF and the relation to the individual with CF (health care provider, parent, spouse or other loved one). It highlighted MI skills including reflective listening, emotion validation, collaborative problem solving and affirmations of effort and values.

Research on the feasibility and benefits of offering brief training in MI to CF health care providers and family members was presented. Several different MI education efforts were piloted with parents of individuals with CF (ages 8-18) including a one-time

seminar, individual training sessions and a web-based support group. With healthcare providers, four brief MI training sessions (~15-20 minutes) were delivered during weekly pulmonology division meetings, plus an optional hour-long skills practice session. Based on the pilot data, benefits and challenges associated with expanding the use of MI to improve treatment adherence in CF was discussed.

Watch Dr. Islam's presentation at <https://tinyurl.com/y6pdq3gc>

### CF Respiratory Infections: non-tuberculous mycobacteria, Small Colony Variant *S. aureus*, and Some Unexpected Consequences of Antibiotic Use

David Nichols, MD  
Seattle Children's Hospital,  
University of Washington

Non-tuberculous mycobacteria (NTM) are a type of bacteria found in the environment and known to infect the airways of people with CF. Because of their waxy coat, relatively slow growth and other characteristics, NTM are difficult to treat with antibiotics. It can also be challenging to determine if these bacteria are causing health problems and when treatment is necessary. Those caring for and studying NTM in people with CF (PwCF) are working to better understand how effective and tolerated current treatment regimens are, and are preparing to study new drug treatment options. Research is also ongoing to better understand the risk of spreading these bacteria between PwCF or in healthcare settings.

*Staphylococcus aureus* is the most common bacteria cultured from the airways of PwCF. Methicillin resistant *S. aureus* (MRSA) is already recognized as a concerning phenotype of this bacteria, but another phenotype is called small-colony variant (SCV), based on the appearance when grown on solid media. Recent research



David Nichols, MD

indicates that SCV *S. aureus*, which can be both sensitive and resistant to methicillin, may be particularly difficult to treat with

antibiotics. It may also be fairly common in US CF centers and most clinical laboratories do not distinguish SCV from other *S. aureus*.

Chronic macrolide therapy in the form of azithromycin, often taken three times a week, is one of the most common chronic pulmonary medications used by PwCF. Clinical trials most strongly demonstrate benefit for those with *P. aeruginosa* infection. This bacterial infection is very commonly treated with inhaled antibiotics such as tobramycin (TOBI). We reviewed research indicating that azithromycin reduces the ability of TOBI to effectively kill *P. aeruginosa*. We also explained what is known, and not yet clear about the health impacts when both drugs are used together in PwCF. Recent work to understand the long-term (years) effects of azithromycin was also discussed. In this context, we briefly reviewed the shifting patterns of use for inhaled antibiotics in the US and how this complicates future clinical trials testing new drug options.

Watch Dr. Nichols' presentation at <https://tinyurl.com/yykjc8fq>

## Innovations in CFRD Diagnosis and Treatment

Marina Basina, MD  
Stanford University Medical Center,  
Palo Alto, CA

Cystic Fibrosis Related Diabetes (CFRD) is one of the most common complications of cystic fibrosis (CF) and occurs in 20% of adolescent and 40-50% of adult population.



Marina Basina, MD

There are similarities and differences between CFRD, type 1, and type 2 Diabetes. CFRD is associated with decline in pulmonary function. Insulin insufficiency is one of the features of CFRD which has been shown to compromise nutritional status in CF.

Elevated blood sugars in uncontrolled CFRD predisposes CF individuals to various infections. Insulin is the best and standard therapy for CFRD. It is demanding and adds more complexity to the daily pulmonary therapies. There are specific factors complicating CFRD

management that include pancreatic insufficiency leading to nutrient malabsorption, chronic infections and CF exacerbations.

Use of diabetes technology may help reduce the burden of diabetes treatment, improve well-being, help with reduction of glucose variability and minimize the risk of low blood sugars from insulin therapy. The available technology, history of its development and the use in CFRD was also discussed.

Watch Dr. Basina's presentation at <https://tinyurl.com/yy5kwrjt>

## Ins and Outs of Gastrointestinal Issues in CF

Mary Abigail Garcia, MD  
UC San Diego / Rady Children's Hospital,  
San Diego, CA

Cystic fibrosis is a genetic condition characterized by impaired chloride and bicarbonate transport via the CFTR ion channel. As a result, defective mucus secretion occurs causing clogging of ducts and lumens in different organs of the body. This presentation focused on the effects of CF in the gastrointestinal tract, particularly on the diagnosis and management of the different gastrointestinal conditions that afflict CF patients.

Abdominal symptoms – lack of appetite, loss of taste, abdominal pain, flatulence and distension – are very common in CF. GI complaints usually arise early in the course of the disease and can have a severe impact on the quality of life of the patient. GI issues have significant influence on disease outcomes, including but not limited to growth and nutrition, pulmonary function and patient-perceived wellness.

Pancreatic insufficiency, which affects up to 85% of all people with CF, and Gastroesophageal Reflux Disease (GERD) are common GI symptoms with CF. The presentation discussed their symptoms and consequences, as well as diagnosis and various treatment options. Less common and more severe GI complications include Distal Intestinal Obstruction Syndrome (DIOS) and gastrointestinal cancers. The



Mary Abigail Garcia, MD

presentation also touched on CF-related liver conditions, as well as the influence of nutrition on GI symptoms.

Watch Dr. Garcia's presentation at <https://tinyurl.com/yxbfwjss>

## Breathing Happiness

Travis Flores, Los Angeles, CA

I have cystic fibrosis, and I am dying of chronic organ rejection of my second double-lung transplant... but I'm also the happiest I've ever been. How? It's a super-power. One that everyone has, but most don't realize it until the end of their life. I am going to help you discover that power now, so that every single day of your life is fulfilling. So, when you wake up you are happy, and when you go to bed you smile at all of the amazing things in your life. When you dream it'll be of happy things, and throughout your life you'll be able to help others unleash their inner superpower as well.

Being sick sucks. Dying? It isn't a picnic in the park. There are a lot of stressors that healthy people deal with on a day-to-day basis, so when you add a terminal prognosis into the mix... life seems to become a bit clearer. The things that matter most to the majority of people, i.e. social media, number of followers or likes, money, religion etc. it all fades away. What we are left with is a choice: Be happy or be miserable.

When so much is negative, misery is the easy route. A lot of people choose that, and who can blame them? Especially when everything feels like it's against you. The superpower though is our ability to choose happiness over misery in the worst situations. It can come in various forms; laughter, smiling, love, joy, gratitude. Happiness comes from within, and with every moment of negativity we encounter, we have the opportunity to find happiness instead, by reminding ourselves of the choice we always have the power to make.

I'm dying laughing... because I choose to be happy in this moment, as I face death.



Travis Flores

Watch Travis' presentation at <https://tinyurl.com/y3eoc8dr>

## CF Summer Retreat: Looking Forward to Next Year! — Georgia Brown

I had heard about CFRI's CF Summer Retreat for adults with cystic fibrosis (CF), but I had always been reluctant to go. Not because of cross-infection concerns, but because I was afraid it would not compare to my experiences attending camp as a kid. I was wrong: I should have gone sooner — much sooner!

I was fortunate to receive a scholarship to spend a wonderful week at beautiful Vallombrosa Retreat Center in Menlo Park, California. It had been so long since I met others with CF in-person, not via a computer screen, that I was nervous. But I soon felt welcome and included.

I know readers may wonder about cross-infection protocols. Did I feel safe? YES! Cross-infection protocols were explained and enforced. Everything was wiped down

before and after each activity. There were plenty of masks, tissues, baggies, gloves and hand gel.

I was assigned my own room with a private bathroom. Volunteers — with sanitized hands — served meals to those with CF. It was hard having to ask for help with food and drinks but it provided a safe space for all attendees. The “six-foot” rule was applied, and windows and doors were kept open in meeting rooms.

Attending the retreat enabled me to connect with the CF community in a way I have not since I was a teenager. The planned activities were fun, and best of all there was time for treatments and resting. We had daily facilitated talks to discuss relationships, women's health, balancing daily routines, and the importance of selfcare.



There were expert talks on sleeping, mental health and GERD. We hiked, played drums, did yoga and spent time crafting. I did not want the week to end. I am so happy that I attended and cannot wait for next year.



## Move-a-Palooza – Thank You to All Who Participated!

We would like to express our gratitude and appreciation to everyone who participated – sponsors, individuals, and whole families! The event raised nearly \$20,000 for CFRI, and the activities ranged from running, dancing and swimming, to BMX biking and CrossFit. We had fun with this inaugural event, and we hope you did too! *Move-a-Palooza* was sponsored by Vertex Pharmaceuticals, Alliance Rx Walgreens + Prime, Chiesi USA, Corbus Pharmaceuticals, Eloxx Pharmaceuticals, Foundation Care, and Translate Bio.



## Hear the Stories of our CF Community, and share yours!



CF Community Voices is a video podcast series created by and for the cystic fibrosis community. Podcasts have focused on CF and mental health, CF and reproductive health, hemoptysis, grieving, advocacy, CF-related diabetes and many other topics.

Episodes are available on CFRI's podcast site, [cfri.podbean.com](http://cfri.podbean.com), as well as on CFRI's YouTube channel.

CF Community Voices is sponsored by Vertex Pharmaceuticals, Chiesi USA, 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

# In Honor of

March 15, 2019 — September 30, 2019

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# In Memory of

March 15, 2019 — September 30, 2019

Kimberley, Marcus and Carol Adelman The Adelman Family Gianna Rose Altano Gary Anderson Jay Archibald David and Jodi Armknecht Anne C. Beltrame Andrea Jamie Bender Brett Bennett Kristina Benson Kenneth A. Berndt Patricia Berndt Amy Bienenstock Wendy Davis Bosarge Mike Bradeson Greg Brazil Ron Broeske Kyle Butler Kenneth Cady John and Carol Carey Scott Carlstrom Sonya Chartrand Cassandra Cochran Ryan Coelho Kelly L. Colgan Martha Colla Irene Colonna	Rachel Crocker Pat Daune Neva L. DeVore Patrick Divine Robert P. Divine Jason Dolan Kerri Efrid Susie Ellerson Ted Ferguson Jessica A. Fredrick Chelsea Fulk-Clark Theresa, Rita-Jean and Cathy Gaspari Jenise Giuliano Royce Goertzen Beverly B. Goulette Eleanor Grammer Christina Groleau Cynthia L. Haley Stephanie Halling David Hardy Mary Hehir Bernyce Helbing Henry Helmers Nicholas Hollis Don, Tracy and Mark Holmes Patsy Howard Robert Huntzinger	Christopher Ireland Jenny Mary K. Jones Linda Junge Sean Kenney Lori Kipp Bridget and John Klein Eleanor Kolchin Jason Konkell Stephanie Lynn Kossoff Jane Ellen Kulik Grant Lindee Dawn Longero Alyson Lowery Johnny Lowery Bob and Nadine Mackey Lucy Marsh Catharine Martinet Priscilla Martiney David McAfee Clifton E. McGee Jennifer McKenna Humberto and Manuel Medeiros Karen Melvin Jessica Mobley Lynn Moyse Dominic Narcisco	Elizabeth Nash Kate Nelson Jolene Nichols Ashley Nowlin Danny and Kevin O'Brien Michele D. Olson Jennifer Ortman Melissa Pappageorgas Lisa Pearne Scott Petersen Kevin Pira Jon and Tim Prater Stephen Primack Catherine Rawlings Pamela K. Rockhold Tom Rolefson Ron Rosenberg Brandon Rossi Mary Roy Billy Ruffner Randy Rupracht Harold Ryder Lucille Scalora Dhea Schalles Jeffery A. Seale Joseph M. Sinnaeve Andrew Sinshack Tammy Smerber	Anabel Stenzel David and Laurie Stuckert Denison Taylor Erin Phillips Taylor Tara Telford Pat and Frank Thibault The Thibault Family Roxanna Thomas June Thompson Dresden P. Tingley Lisa Tingley Louis Anthony Trigueiro Betty Lee Trolan Frieda and Frank Turner Jennifer Uskoski Rory and Jerry Vaeth Tom Walton Nicky Wastell Viola Wastell Tara Weir Hayley C. Wester Chris Williams Cynthia Witman Gertrude Zumwalt
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# 35th Annual Golf Tournament Benefitting CFRI – Another Successful Event!

The legendary Pasatiempo Golf Club in Santa Cruz, CA – a “Top 100” course – hosted 152 golfers on August 12, who enjoyed friendly competition while supporting the search for a cystic fibrosis (CF) cure. The 35th annual benefit event for CFRI raised approximately \$75,000 in support of CF research! Of this total, \$15,000 will be matched by CFRI’s Jessica Fredrick Memorial 2019 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. Scott, General Manager of Pasatiempo Golf Club and former CFRI Treasurer, has two daughters with CF, one of whom received a

life-saving double lung transplant in 2016. Mike, former member of CFRI’s Board of Directors, is the father of Becca, who lives with CF. Star One Credit Union was the Tournament Sponsor, while the Mike and Dea Roanhaus family and the Kirkorian Family Foundation were Executive Sponsors.

CFRI is extremely grateful to Scott, Mike, and the other dedicated members of the golf committee, Francine Bion, 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. Save the date for the 36th annual tournament, which will be held August 10th, 2020.



Team Erinn and Kristen 2019



## CFRI and You: Partners for Life

*Some Easy Ways to Deepen Your Relationship*

- **COME to a CFRI FUNDRAISING EVENT**, or hold your own! Many community members create fundraisers for CFRI by donating their birthdays on Facebook. Check out our listings at [cfri.org](http://cfri.org), or think “outside the box” and we’ll help you make it happen.
- **TRIBUTES:** “In Honor Of” and “In Memory Of.” Recognize a loved one with your choice of gift. CFRI will send an acknowledgement letter to your designee.
- **DONATE A CAR, MOTORCYCLE OR BOAT:** Vehicle move will be arranged and you will receive paperwork for a tax deduction. Remember to designate Cystic Fibrosis Research, Inc. (CFRI) as your charity so that the proceeds can go to CFRI programs. For more details, go to the “Ways to Give” section on our website.
- **MONTHLY GIVING PROGRAM:** *Champions of Hope* supports research to find a cure for CF and enhance CFRI’s programs in CF education, support and advocacy.
- **GIVE STOCK 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. Your designated amount would come to CFRI - tax-free to your heirs and CFRI.

For more information, please contact Mary Convento at 650.665.7559 or [cfri@cfri.org](mailto:cfri@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  
Nov. 19 • Dec. 17 • Jan. 21  
Feb. 18 • March 17 • April 21

Go to [www.cfri.org](http://www.cfri.org) for information.  
Participate in person or by phone  
Sponsored by Vertex Pharmaceuticals and Gilead Sciences

### Online Support Group for Adults with CF

Third Monday of Every Month  
Nov. 18 • Dec. 16 • Jan. 20  
Feb. 17 • March 16 • April 20

Go to [www.cfri.org](http://www.cfri.org) for information.  
Participate in person or video conference

### Embrace Mothers’ Retreat

May 1 – May 3, 2020  
Vallombrosa Center, Menlo Park, CA

### CF Summer Retreat

July 26 – July 31, 2020  
Vallombrosa Center, Menlo Park, CA

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

July 31 – August 2, 2020  
Pullman San Francisco Bay  
Redwood City, CA

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



## Bacterial Viruses in Cystic Fibrosis Lungs

Continued from front cover

increase bacterial pathogenicity. In one case, a phage infecting *Stenotrophomonas maltophilia* encoded the toxin zot, capable of direct damage to the lung tissues. This phage was actively replicating in the patient's lung, and the viral particles were detected at high abundances. In another case, a phage genome region was identified in the genome of *Achromobacter xylooxidans* encoding a virulence gene that enables bacterial adhesion to human epithelium and evasion from the immune system. Given the individualized nature of the microbial assemblages

in the lungs of each patient, the CFRR efforts are fundamental to reveal cases where phage transfer of genes is involved in bacterial virulence and for the identification of potential targets for phage therapy.

With ongoing support from CFRI's Elizabeth Nash Memorial Fellowship and a recent New Horizons award, we are focusing on advancing strategies that can direct phage lysis for bacterial control. Two strategies have generated promising results: the isolation and production of novel phages infecting *Achromobacter*, which are now available for use in phage therapy, and the engineering of tailocins targeting *Stenotro-*

*phomonas*. Tailocins are components of the phage structure capable of killing bacteria while avoiding the release of toxins and the risk of phage transfer of virulence genes, which are potential side effects of bacterial cell lysis. The tailocins can be modified to target specific pathogens; our goal is to build tailocin scaffolds that can be adapted to the different pathogens in each patient. The combination of phage-based strategies with conventional antimicrobial therapies may be the most effective method to control bacterial growth in the short-term, a much-needed solution for adult patients with chronic lung infections.

## CFRI's Moonlight Masquerade A Phenomenal Success!

CFRI's annual gala, held at the elegant and historic Hillsborough Racquet Club on October 26, 2019, brought together 135 champions united in the search for a cure for cystic fibrosis. Warm thanks to Barbara and Jim Curry, who graciously co-hosted the evening at the Racquet Club. Our emcee, Chris Chmura of NBC Bay Area, kept the evening lively. Tiffany Raffino, LCSW, of UCSF, was honored as our 2019 CF Champion, while Rohini and Richard McKee shared their journey as parents to Ria, four years old and living with CF. The live and silent auctions raised vital funds for CF research, with spirited bidding on enticing packages generously donated by numerous businesses. By the



Rohini, Ria and Richard McKee



Tiffany Raffino, LCSW



end of the evening, nearly \$90,000 was raised to support CFRI's research, education and support programs. We also welcomed back the Latin duo "IndiviDúo," who had everybody on their feet dancing away this magical night.

We are grateful for our generous sponsors, in-kind donors, attendees and hardworking Gala Committee members. Everyone played a role in our Moonlight Masquerade's amazing success!

### Moonlight Masquerade Sponsor —

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Members of CFRI's Jessica Fredrick Memorial CF Research Challenge Circle match – dollar for dollar – donations from individuals committed to CF research. These combined

## Jessica Fredrick Memorial CF Research Challenge Circle and Fund

donations are used for our New Horizons and Elizabeth Nash Memorial Fellowship CF research awards.

Our Circle is named in memory of Jessica Fredrick, pictured, who lost her battle with CF at the age of 21. Please join the inspiring members 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.

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. If desired, you can use the envelope in this newsletter. Despite tremendous progress in CF therapies, we continue to lose our loved ones to this cruel disease, and there is still no cure.

Please give today. By giving all to the present, you are generously supporting the future hopes of those with CF.



# Recognizing Our Extraordinary Community Heroes

CFRI is proud to honor the following people who have made extraordinary contributions to the cystic fibrosis (CF) community. Awards were presented at our 32nd National CF Education Conference on July 27th, 2019.



The 2019 David Stuckert Memorial Volunteer of the Year Award was presented to **Francine Bion**. Over the past 30 years, Francine has generously shared her time and expertise with CFRI in a variety of ways. She volunteered with CFRI after her niece, Lauren, was diagnosed with CF, and has been pivotal in welcoming other extended families to join the fight against CF. Through the years she has served on numerous committees, including Newsletter, Gala and Strategic Planning. She currently serves on CFRI's Board of Directors, Conference Committee and the CFRI Benefit Golf Tournament Committee. Francine

exemplifies the passion and devotion of our long-time volunteers at CFRI.

The 2019 CFRI CF Professional of the Year Award was presented to **Carole Nakamura, MSN, RN, PCCN, CMSRN**. Ms. Nakamura brings decades of service to CFRI and the cystic fibrosis community. As a unit educator and clinical nurse on the medical units at Stanford Hospital, she has coordinated several CF nursing continuing education courses at Stanford over the past 20 years, and is currently a Nursing Professional Development Specialist (NPDS) at Stanford. Ms. Nakamura brings her compassion, energy and expertise to many CFRI events. She volunteers for CFRI as a Medical Advisor for the annual education conferences, as well as at CFRI's CF Summer Retreats. She also shares countless hours each year overseeing CEU accreditation for eligible CFRI conference attendees.



The 2019 CFRI Partners in Living Award in Memory of **Anabel Stenzel** was awarded to **Jean Hanley, MD**. Dr. Hanley is an adult with CF who is now in her fifties and thriving. An allergist-immunologist in Manhattan Beach, CA, she has been in practice for more than 20 years, and is also the President of the US Adult Cystic Fibrosis Association (USACFA). She was diagnosed at age 33 after many years of CF-like symptoms treated as "refractory asthma." Her late diagnosis came as a big relief as she could finally receive the proper care, treatment and support services. She has written numerous



articles for CF Roundtable, and since 2012 has also run a non-profit Patient Advocacy Service, called Planning Health at [planninghealth.org](http://planninghealth.org).

The 2019 Paul M. Quinton Cystic Fibrosis Research Legacy Award was awarded to **Alan Verkman, MD, PhD**, a living legend in the field of cystic fibrosis research. Dr. Verkman has worked in CF research for more than 25 years, and directs cystic fibrosis research programs at University of California San Francisco that are funded by the National Institutes of Health and the CF Foundation. His main interest in CF research is in the development of new drug strategies and the study of CF lung disease mechanisms. Dr. Verkman was responsible for the original concept of potentiators and correctors of mutant CFTR in the late 1990s, for which he received the inaugural Robert J. Beall CF Therapeutics Development Award in 2015. Dr. Verkman's recent research focuses on drug development for cystic fibrosis patients with relatively uncommon mutations that cannot be treated with current CFTR modulators



**Congratulations to these extraordinary CF community heroes!**

# CFRI's 32nd National Cystic Fibrosis Education Conference: Charting the CF Course

July 26 – July 28, 2019

Thank you to our generous sponsors & exhibitors who made this event possible!

The image displays a collection of logos for various pharmaceutical and healthcare companies. The logos are arranged in a grid-like fashion. The companies included are: Genentech (A Member of the Roche Group), GILEAD, abbvie, IONIS PHARMACEUTICALS, VERTEX, Chiesi (People and Ideas for Innovation in healthcare), pti PROTEOSTASIS THERAPEUTICS, INC., allianceRx (Walgreens + PRIME), ELO (Eloxx Pharmaceuticals), Foundation Care (An AcariaHealth Solution), Kroger (SPECIALTY PHARMACY), Allergan, Translate BIO, PARI, RARE PATIENT voice, santhera, MAXOR (National Pharmacy Services Company), ROCK CF, CORBUS PHARMACEUTICALS, MED SYSTEMS, RespirTech, and ALCRESTA THERAPEUTICS.



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## 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 and Chiesi USA**

Visit our website at:  
**www.cfri.org**

for more information about us and about cystic fibrosis.

Call toll free: **855.cfri.now**

## CF Quality of Life Programs

### CF Caregivers Support Groups

CFRI provides two monthly Caregiver Support Groups, which are open to participants nationwide. Facilitated by a CF social worker, the groups meet the third Tuesday of the month in person and via Zoom.

### Online Support Group for Adults with CF

CFRI provides a monthly online Support Group for Adults with CF, which is open to participants nationwide. The group is facilitated by a social worker well versed in issues facing adults with CF. The group meets on the third Monday of every month, from 6:00 pm PST to 7:30 pm (9:00 pm – 10:30 pm EST).

### Living Mindfully with CF

CFRI's online 'Living Mindfully with CF' class – created and taught by Julie Desch, MD, who herself has cystic fibrosis - draws upon Mindfulness-Based Stress Reduction techniques and focuses these practices specifically to living with CF. Classes are free, and open to individuals with CF and their family members (spouses/partners, parents, siblings) from across the United States and beyond. Participants must be at least 16 years old.

### Counseling Support Program

Children and adults with CF as well as their family members (parents, siblings, spouses/partners) can enroll in individual therapy sessions with a licensed provider of their choice. CFRI will cover up to \$120 per session for six sessions (either your co-pay, or your therapist's fee).

**For more information** on any of these programs, please go to our website at [www.cfri.org](http://www.cfri.org).

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

