cfri community

43 Years of Research,
Education & Support

Fall 2018

CFRI's 31st National Cystic Fibrosis Family Education Conference: Speaker Abstracts

At CFRI's 31st National CF Family Education Conference, Bridges to the Future, held August 3 to 5, 2018, nationally-renowned speakers from across the country shared their expertise and experience on a wide range of CF-related topics. Nine 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.

Save the Date! CFRI's 32nd National Cystic Fibrosis Education Conference will be held July 26 – July 28, 2019!

CF Pipeline: The Journey Continues

Manu Jain, MD —
Northwestern University School of Medicine

Life expectancy in cystic fibrosis (CF) has improved substantially over the last 75 years, with a median predicted survival of approximately 47 years. This has been due for the most part to therapies that have targeted lung disease and other end organ manifestations in an attempt to disrupt the cycle of mucous obstruction, inflammation, and infection. In an effort to develop drugs that would target the underlying defects in the CF transmembrane conductance regulator (CFTR), the Cystic Fibrosis Foundation embarked on a bold initiative in which it established collaborations with biopharmaceutical companies to support early-stage efforts to discover new medicines for CF. Over the past decade the focus of emerging therapies has shifted toward discovering drugs that target the underlying genetic protein defects in CF, collectively referred

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Dr. Kenichi Okuda

Localization of Secretory Mucins MUC5AC and MUC5B in Normal Human Airways

Kenichi Okuda, MD — University of North Carolina

Rationale: Mucin secretion is one of the key components of the mucociliary clearance (MCC). Dysregulated mucin secretion can produce MCC dysfunction and worsening of chronic lung disease including cystic fibrosis. A full understanding of the regional expression of the two major secretory mucins, MUC5AC and MUC5B, in the human lung is critical to elucidate how these two mucins functionally interact in health and during disease.

Objectives: To characterize the regional distribution of MUC5AC and MUC5B in normal human airways and assess which cell types produce these mucins, referenced to club cell secretory protein (CCSP).

Methods: Multiple airway regions from 16 non-smoker lungs without a history of lung disease were studied. MUC5AC, MUC5B, and CCSP expression/co-localization were assessed by RNA in situ hybridization (ISH)

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CFRI Community
Fall 2018

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Letter from the Executive Director

Dear Friends,

We have experienced a significant change at CFRI this year. Sue Landgraf, our intrepid leader since 2013, retired last May only to have another door open that allowed her to pursue a lifelong dream of restaurant ownership. I am honored to now serve as CFRI's executive director. I discovered CFRI shortly after my daughter Tess' diagnosis with cystic fibrosis (CF) in 1995, and since then have been involved in many capacities, including as a 10-year member of the Board of Directors, and staff member since late 2013. I love CFRI and am wholeheartedly committed to its continued responsive growth.

In recent months, CFRI expanded its funding for innovative CF research. Our groundbreaking CF Quality of Life programs, National CF Education Conference, and nationwide CF advocacy and awareness efforts flourish, while our new online "Breathe In ~ Work Out CF Wellness Initiative" for the international CF community was an instant success.

Most recently we hosted an Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis in Hyattsville, Maryland, which brought the patient voice to representatives of the FDA. This momentous and interactive event was live-streamed, and attendees from across the U.S. played a vital role in illuminating the ongoing challenges and tragedies of CF, and our community's urgent need for new therapies.

Many individuals, foundations and corporate sponsors have made these programs possible. I encourage you to engage with CFRI. 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. I am delighted to share that CFRI's leadership transition has been seamless. Sue and Siri worked closely together for many years to expand and enhance CFRI's programs. Siri brings years of experience to her new role, including as a nonprofit executive, grant writer and social worker. We are in good hands.

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 $1.6 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 Annual Report demonstrates the impact and reach of these key programs. Our effectiveness and efficiency has led to CFRI proudly receiving another 4-star rating from Charity Navigator.

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

Bill Hult | *President,* CFRI *Board of Directors*

Manu Jain, MD

to as CFTR modulators. Work by basic scientists has improved understanding of the effects of specific mutations on CFTR protein function and as a result, a system to group CFTR mutations has been developed. In general CFTR mutations are categorized into six classes depending on the specific defect protein synthesis, transport, or function. This has led to the development of novel drugs which target specific defects, the first two of which were CFTR potentiators and correctors. At this time three drugs have been approved for specific CFTR genotypes, which provide CFTR modulator options for nearly 60% of patients. However, this still leaves almost 40% of patients without modulator options while the options available for many patients have modest benefit and significant side effects.

To address these limitations, a triple combination of 2 complementary correctors and a potentiator was developed and data from recent early phase clinical trials suggest that this combination may provide a highly effective CFTR modulator option for nearly 95% of all CF patients. In addition, theratyping, where a mutation can be matched to a medication, may identify therapeutic options for the remaining patients with rare mutations. In addition, RNA editing or replacement offer genotype independent options to increase CFTR protein. Further down the road the concept of a "cure" could be possible with developments in gene therapy, DNA editing or stem cells.

In addition, other research avenues being explored include novel anti-inflammatory drugs, antibiotics and optimizing lung transplantation. In summary CF care has come a long way, but much work remains as the journey continues.

Watch Dr. Jain's presentation at https://tinyurl.com/y92jm5v3

Top: Jasleen Kukreja, MD
Bottom: Rupal Shah, MD

Advances in Lung Transplantation

Jasleen Kukreja, MD & Rupal Shah, MD — UC San Francisco

While life expectancy has improved significantly for those living with cystic fibrosis (CF), lung transplantation remains the only therapeutic option for those with very advanced lung disease. Early identification of appropriate patients for transplant evaluation and timely referrals to a transplant center are critical to success. Infectious complications can add a unique complexity to the management of a lung transplant recipient with cystic fibrosis. The medical and surgical approach to difficult to treat infections was discussed during this session. Extracorporeal membrane oxygenation (ECMO) technology was discussed as a vital tool for many patients waiting for transplant, while the Breathing Lung currently in development may someday expand the number of usable organs available for transplant. Drs. Kukreja and Shah also reviewed the evaluation process, peri-operative management and outcomes after lung transplant for recipients with cystic fibrosis.

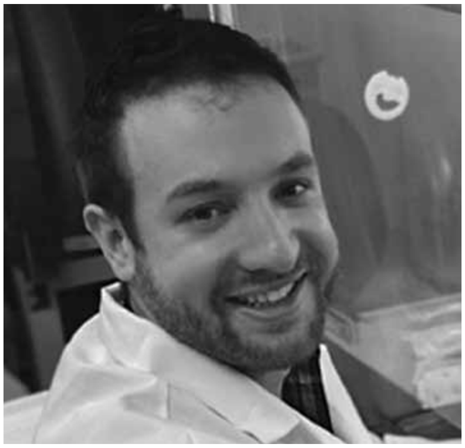
Watch Dr. Kukreja and Dr. Shah's presentation at https://tinyurl.com/yc4gh5u7

Science, Advocacy & Life with CF

Reid D'Amico, PhD —
US Food and Drug Administration

As a member of the cystic fibrosis (CF) community, Reid discussed his personal experience as an adult with CF, along with his professional experiences as a biomedical engineer, advocate, and incoming scholar at the US Food and Drug Administration. Reid shared how CF can be used as a foundation for advancing insight in the fields of engineering and science. In particular, Reid discussed how being an adult with CF allows him to experience the world differently, and ultimately influences his ability to innovate and create policy. Key objectives of Reid's talk included: providing an overview of the psychological and physical challenges often faced by members of the CF community in the lab or workplace; using a CF identity to challenge the status quo; discussing how the CF community can teach others to be agents of change in their personal and professional lives.

Watch Reid's presentation at https://tinyurl.com/ybnz9vxv



Reid D'Amico, PhD

Fungi in CF: Observers or Actors?

Richard Moss, MD — Stanford University

Fungal infection in cystic fibrosis is a recognized challenge, with many areas requiring further investigation. Consensus definitions

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exist for allergic bronchopulmonary aspergillosis (ABPA) in CF, but the full scope of clinically relevant nonallergic fungal lung involvement in CF is still poorly understood. The possibilities range from asymptomatic and harmless colonization, to transient or chronic local airways infection, to local or widespread invasive disease — and all of these are yet to be clearly defined. Recent advances in fungal culture, and non-culture molecular identification of fungal species DNA, have expanded the list of both potential disease-causing agents (pathogens, "players") and community commensals (harmless colonizers, or "observers") in the lower respiratory tract well beyond the most common and best recognized pair, *Aspergillus fumigatus* and *Candida albicans*.

In his presentation, Dr. Moss outlined **[a]** the current understanding of the prevalence and diversity of fungal presence in the CF respiratory tract, **[b]** some risk factors for acquiring fungi, **[c]** some of the body's responses ("host factors") that influence whether a fungus is a commensal bystander or a dangerous player, **[d]** advances in the diagnostic approaches to isolating and identifying fungi in CF respiratory samples, **[e]** challenges of classifying clinical presentations and problems ("phenotypes") of CF patients harboring fungi, and **[f]** current treatment approaches. Development and validation of biomarkers characteristic of different fungal clinical phenotypes, and controlled trials of anti-fungal agents in well-characterized target populations, remain central challenges to surmount and goals to be achieved.

Watch Dr. Moss's presentation at https://tinyurl.com/y9p396hm

Connecting the Dots: Bridging the Gap Between Isolation and Social Connection

Chelsea Toth, DSW — Kutztown University

Rick Moss, MD

While cystic fibrosis (CF) was once known as a childhood disease, given advances in medical technology, individuals with CF are living into adulthood. Historically, individuals living with CF would participate in activities with others diagnosed with the disease by attending summer camps, community events, and support groups. However, current medical guidelines recommend strict separation of individuals with CF to reduce the risk of cross-infection. Dr. Toth presented the results of a study that examined the impact of these guidelines on young adults with CF. A qualitative, exploratory, grounded theory approach was utilized to understand the experience and the impact of social isolation among young adults living with CF. Semi-structured individual interviews were conducted with ten individuals aged 18 – 25 and diagnosed with CF. During the interviews, information was elicited about how individuals with CF understand, experience, and perceive social isolation from others with CF; ways in which they experience isolation; and the impact isolation has on their medical and psychosocial development. The results identified the importance of social connection and isolation as a common theme supported by participants' level of connection with others. The experience of living with an invisible illness was discussed by participants along with avenues they utilize to connect with peers, family, and their community. Identifying barriers to isolation and discussing the importance of connection is key to developing networks for individuals living with CF and their families.

Chelsea Toth, DSW

Watch Dr. Toth's presentation at https://tinyurl.com/y99tgj9w

Ouch! Living with Pain in Cystic Fibrosis

Elaine Chen, MD —
Rush University Medical Center

Both acute and chronic pain are common in people with cystic fibrosis (CF), and can significantly impact quality of life and adherence to therapies. In this session, Dr. Chen described various causes for pain with CF, including those related to complications of CF, treatments for CF, or those that are unrelated to CF. The experience of pain is complex, and is related to much more than simply the physical aspects of pain. The concept of "Total Pain" was first described by Dame Cicely Saunders, the founder of modern hospice in the UK in the 1960's. While this concept was developed in the setting of hospice and end-of-life, it can be applied to the broader setting of chronic illness such as CF. The concept of Total Pain includes four elements of pain - Physical, Social, Spiritual, and Psychological, each of which was addressed by Dr. Chen.

Elaine Chen, MD

Strategies for managing pain include a multidisciplinary approach targeting each aspect of Total Pain described above. In addition to adjunctive therapies for addressing pain, Dr. Chen also discussed the benefits and risks of different classes of medications which can be considered in the treatment of pain. An approach to the use of opioids in

CF was outlined. Risks of the use of opioids include medication side effects such as constipation and confusion, misuse and abuse, and societal stigma. Dr. Chen delved into challenges related to the management of pain that are specific to CF, such as respiratory function and the possibility of lung transplantation. The objective of the presentation for audience members was that they gain a greater understanding of the many complexities underlying pain management in CF, and approach their own pain and that of their patients and/or loved ones with an open mind.

Watch Dr. Chen's presentation at https://tinyurl.com/y9khhazx

History and Future of CFTR Modulators

Alan Verkman, MD, PhD — UC San Francisco

Following the discovery in 1989 that CF is caused by mutations in the CFTR chloride channel gene, remarkable progress has been made in CFTR-targeted, disease-modifying therapies for CF. CFTR modulator therapy is based on the discovery that the function of most mutant CFTRs that cause CF can be partially restored by small molecules. The first "potentiators" of mutant CFTRs, which rescue their defective chloride channel function, were identified by high-throughput screening and reported by Dr. Verkman's lab in 2003, and in 2005 he and his colleagues reported the first "correctors" of defective cellular processing of the deltaF508 CFTR mutant. At present, one potentiator and two correctors are FDA-approved to treat most CF patients, and, with new triple drug combinations in clinical trials, it may be possible to confer significant benefit to up to 90% of CF patients. Continued advances in CFTR modulator drugs are anticipated as driven by high commercial interest. However, effective CFTR modulator therapy for the "remaining 10%" remains challenging, though recent advances in combination potentiator therapy and CFTR modulator therapy for premature stop codon mutations offer a path forward. In addition, advances in "CFTR-agnostic therapies" that target non-CFTR chloride channels, sodium channels, or SLC26A-family anion transporters hold promise as providing disease-modifying therapy for all CF patients, and over the long-term, gene replacement and editing approaches hold promise.

Watch Dr. Verkman's presentation at https://tinyurl.com/ybfgg8af

Running, Adversity and CF

Sabrina Walker — Anchorage, AK

Cystic fibrosis was once known only as a childhood disease, but now has transitioned into a disease where people are living into adulthood. Sabrina, a native of Anchorage, Alaska, discussed strategies for coping with the unpredictable challenges that arise when living with cystic fibrosis. CF is a progressive disease that impacts patients in a multitude of ways. While staying adherent with medications and airway clearance is essential, Sabrina touched upon exercise as a complementary way to maintain health among those with CF. As a woman with CF who is also a cancer survivor, Sabrina touched upon the reality that many people experience more than one diagnosis. People with CF are now facing new life challenges like planning for the future in terms of college, careers, marriage, and children. As an adult who is married and has a three-year-old son, Leo, Sabrina provided her personal perspective on having children with CF.

Watch Sabrina's presentation at https://tinyurl.com/y8mfa7hc

Top: Alan Verkman, MD, PhD
Bottom: Sabrina Walker

Localization of Secretory Mucins

Continued from front cover

and immunohistochemistry in 5 lungs with histologically normal airways. Droplet digital PCR (ddPCR) was performed for absolute quantification of MUC5AC/5B ratios in different airway regions, including large and small airway epithelia. Large airway epithelial (LAE) and small airway epithelial (SAE) cells were cultured in air-liquid interface condition and utilized for MUC5AC/5B protein secretion.

Results: Submucosal glands expressed MUC5B, but not MUC5AC. However, MUC5B was also extensively expressed in superficial epithelia throughout the airways except for terminal bronchioles. Morphometric calculations revealed that the predominant site for MUC5B production in the superficial epithelium was the distal airways, whereas MUC5AC production was concentrated in large, cartilaginous airways. RNA ISH revealed both MUC5AC and MUC5B were co-localized with CCSP-positive secretory cells in proximal superficial epithelia, whereas MUC5B- and CCSP-co-positive cells dominated distal regions. Absolute quantification of MUC5B and MUC5AC RNA by ddPCR in freshly isolated airway epithelia revealed that MUC5B expression was significantly higher than MUC5AC in distal airways. In vitro study, mass spectrometry identified significantly more abundant MUC5B protein than MUC5AC in apical secretions of SAE cells. RNA ISH showed MUC5B expression in CCSP positive non-ciliated secretory cells in both LAE and SAE cells. These ddPCR and in vitro data were consistent with the findings shown in human airway tissues by RNA ISH and immunohistochemistry.

Conclusions: In normal human airways, MUC5B is the dominant secretory mucin in the superficial epithelium as well as glands, with distal airways being a major site of expression. MUC5B and MUC5AC expression is a property of CCSP positive secretory cells in superficial airway epithelia.

CF Summer Retreat: A Place of Community for CF Adults

By Michelle Cowin-Gantz

Cystic fibrosis doesn't discriminate against people or their organs. I was diagnosed at the age of 48 with CF after struggling for years with horrific, painful, chronic pancreatitis. My mutation is rare and attacks my pancreas and GI tract; however, I'm not exempt from the typical organ involvement such as lungs and sinuses. The pain has become crippling to the point where I am disabled, can no longer work, and became isolated. I looked for ways to find community and connection. When I heard about CFRI's CF Summer Retreat, I knew I had to find a way to attend and meet other adults with the same struggle. I was given a scholarship to attend and headed to California from my home state of Virginia.

What affected me so deeply was finally meeting people who really understood my struggle. Even though I was the new "kid," the community welcomed and embraced me. We openly shared our battles, our scars, our pain and our victories. We could see that even though our "bumps in the road" are slightly different, the beast is the same. Together we share strength, comfort, encouragement and understanding.

At the retreat I switched from "me" to "we," as I realized I am no longer alone; I have a community. I can contact and lean on others who encourage me on my bad days, cheer for me on the good days, and who send notes just because they are thinking about me. If you haven't attended retreat before I strongly encourage it. It has truly changed my life. The only problem I have with retreat is I must wait a whole year to see my friends again!

Hear the Stories of our CF Community

CFRI's CF Community Voices is a video podcast series created by and for the cystic fibrosis community. Each month, two video podcasts are released that address diverse and complex CF-related topics. Podcasts have focused on CF and mental health, CF and reproductive health, hemoptysis, grieving, advocacy, CF-related diabetes, and many other topics.

Podcasts are presented by CF researchers and clinicians, as well as those living with the disease. Episodes are available on CFRI's podcast site, cfri.podbean.com. You can watch or listen at the site, or download to your phone or tablet to enjoy in the car or while exercising. Episodes are also posted 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:
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In Honor of April 1, 2018 — August 31, 2018

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In Memory of April 1, 2018 — August 31, 2018

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Pet Memorials

Cowboy Glass
Sierra Garlow

34th Annual Golf Tournament Benefitting CFRI A Record Setting Event!

The legendary Pasatiempo Golf Club – a “top-100” course – hosted an energetic group of 154 golfers on August 13, who enjoyed friendly competition while supporting the search for a cystic fibrosis (CF) cure. The 34th annual benefit event for CFRI raised over $85,000 in support of CF research! Of this total, $15,000 will be matched by CFRI’s Jessica Fredrick Memorial 2018 CF Research Challenge Circle and designated for CF research grant awards.

The event is deeply personal for the event co-chairs, Scott Hoyt and Mike Roanhaus. Scott, Pasatiempo Golf Club Manager and former CFRI Treasurer, has two daughters with CF, one of whom received a life-saving double lung transplant in 2016. Mike, current 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, Kirkorian Family Foundation, and Living Breath 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 35th annual tournament, which will be held August 12th, 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. Go to https://www.facebook.com/cfri.org/, scroll down to Fundraisers, and click on Create! Check out our listings at cfri.org, or think “outside the box” and we’ll help you make it happen.
- **MONTHLY GIVING PROGRAM:** *Champions of Hope* will provide a predictable 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 Tony Adessa (see below).
- **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.
- **GIVE STOCK to CFRI:** Donating appreciated stock avoids capital gains taxes that you might have incurred had the stock been sold. You’re also entitled to an income tax charitable deduction for the stock’s fair market value on the date of the gift.
- **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 CRFI 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 Tony Adessa, CFRI’s Development Manager: 650.665.7586 *or tadessa@cfri.org.*

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

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Third Tuesday of Every Month
November 20 • December 18
January 15 • February 19
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Go to www.cfri.org for information.
Participate in person or by phone
Sponsored by Vertex Pharmaceuticals and Gilead Sciences

Embrace Mothers’ Retreat

May 3 – May 5, 2019
Vallombrosa Center, Menlo Park, CA

CFRI’s 32nd National CF Education Conference

July 26 – July 28, 2019
Pullman San Francisco Bay
Redwood City, CA

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

CFRI's Externally-Led Patient-Focused Drug Development (PFDD) Meeting on CF Brings the Voice of the Community to the FDA

Held Monday, October 29, this momentous event integrated the patient voice into the drug development and review process during a one-day live-streamed meeting of individuals with CF, parents/caregivers, clinicians, and FDA representatives. The day included FDA representatives, and a slate of speakers who shared their knowledge and experience living with CF. Boomer and Gunnar Esiason, Dr. Ahmet Uluer, Isabel Stenzel Byrnes, Emily Kramer-Golinkoff, Emily Schaller, and other articulate and passionate members of our CF community helped FDA representatives to better understand the impacts of the disease, the burden of care, the need for new therapies, and our community's willingness to take part in the advancement of drug development.

The event was live-streamed, and all participants, whether online or in the room, had the opportunity to express their views. Stay tuned for the release of the recorded proceedings – CFRI is still collecting our community's individual and collective experiences with cystic fibrosis. We want to incorporate your voice so that those evaluating new therapies will understand our urgent need.

Sponsors: ***Platinum*** – *Vertex Pharmaceuticals;* ***Gold*** – *Gilead Sciences;* ***Silver*** – *Genentech, Ionis Pharmaceuticals, Proteostasis Therapeutics, and Synspira;* ***Bronze*** – *AzurRx BioPharma, Eloxx Pharmaceuticals, Mylan Pharmaceuticals (through an independent medical educational grant), Santhera Pharmaceuticals and Translate Bio.*

CFRI's Enchanting Adventure at Nestldown a Phenomenal Success!

CFRI's annual gala, held at the breathtakingly beautiful Nestldown estate, brought together 155 champions united in the search for a CF cure. Warm thanks to Barbara and Mark Beck, who graciously donated the use of their amazing destination property. Chris Chmura, of NBC Bay Area, emceed the evening. Paul Mohabir, MD, was honored as our 2018 CF Champion, while Arek and Marta Puzia shared their journey as parents to Natalie, 4 years old and living with CF. Gina Serrato introduced the Latin duo "IndiviDuo," who debuted a new CF anthem for a cure, "The Way I See the World," co-written by Gina's 8-year old daughter Gianna, who is living with CF. The auction raised vital funds for CF research, with spirited bidding on incredible packages generously donated by numerous businesses. By the end of this magical evening, over $96,000 had been net to support CFRI's research, education and support programs.

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

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Mark & Barbara Beck, *Nestldown*

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CFRI Support Groups
cfri Cystic Fibrosis Research Inc.


July 26 – 29, 2019
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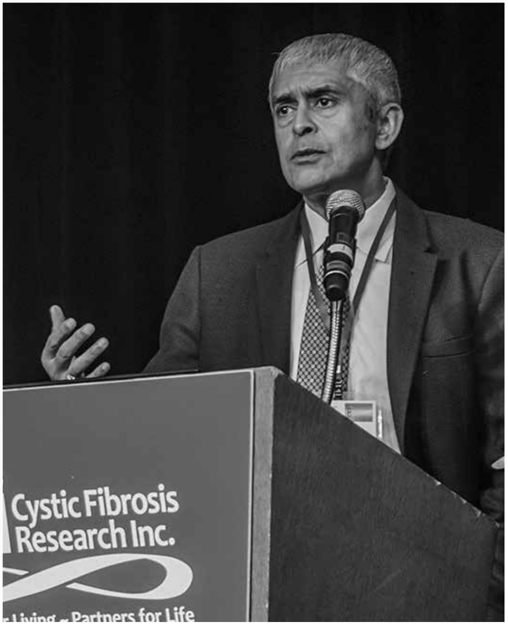
Cystic Fibrosis Research Inc.
Living ~ Partners for Life

CFRI's 31st National Cystic Fibrosis Family Education Conference:

Bridges to the Future

August 3 – August 5, 2018

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

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Recognizing Our Extraordinary Community Heroes

By Siri Vaeth, MSW

CFRI is proud to honor the following people who have made extraordinary contributions to the cystic fibrosis (CF) community. Awards were presented at our 31st National CF Family Education Conference on August 4th, 2018.

The 2018 David Stuckert Memorial Volunteer of the Year Award was presented to **Danielle Mandella**. Danielle is an inspiring woman with CF who serves on CFRI's CF Summer Retreat Committee, where she helps select speakers and plays a key role with event marketing, including creating the Retreat "Buzzguide," for event fundraising and promotion. Danielle has participated at CFRI's advocacy days in Sacramento, where she educated her elected representatives about CF and issues facing the CF community.

The 2018 CF Professional of the Year Award was presented to **John Mark, MD**. A pediatric pulmonologist at Lucile Packard Children's Hospital Stanford, Dr. Mark has cared for countless children with CF through the years. In 1999, Dr. Mark completed the first fellowship in Pediatric Integrative Medicine ever funded by the National Institutes of Health, and he integrates the mind/body approach to healing as a complementary approach with standard CF care. Dr. Mark is the Program Director for the Pediatric Pulmonary Fellowship Program, Associate Director for the Pediatric Residency Program and the Medical Director for the CORE program, which assists with care coordination for children with complex medical needs. Dr. Mark regularly shares his time with CFRI, including multiple presentations for CFRI conferences, Discovery Series, and CF Community Voices podcasts.


From top left, clockwise: Danielle Mandella, John Mark, MD, Devin Wakefield, Jeff Wine, PhD

The 2018 CFRI Partners in Living Award in Memory of Anabel Stenzel was awarded to **Devin Wakefield**. Devin has served on CFRI's CF Summer Retreat Committee for many years, where he records Retreat Committee minutes, actively markets the Retreat, holds Google hangouts to promote the event, and helps with all aspects of Retreat planning. He has served as an office volunteer, serves on the Research Advisory Committee, participated on a CFRI conference panel, and raises funds for CFRI, particularly through his workplace giving at Microsoft.

The 2018 Paul M. Quinton Cystic Fibrosis Research Legacy Award was awarded to **Jeff Wine, PhD**, Benjamin Scott Crocker professor of Human Biology and Director of the Cystic Fibrosis Research Laboratory at Stanford University. His research in cystic fibrosis was sparked by his daughter's diagnosis with CF in 1981. Since 1987, Dr. Wine has studied genetic and cellular aspects of CF in humans and animal models to increase understanding of the disease in ways that will improve treatment. He has been a Principal Investigator in numerous research projects funded by NIH, CFF, CFFT, CFRI's Elizabeth Nash Memorial Fellowship Program, and CFRI's New Horizons Program. Dr. Wine and his colleagues identified the first heterozygote effect of the CFTR mutation, and he has recently adapted that assay to accurately measure levels of CFTR function that are restored by CFTR-directed therapies. His studies of the innate defense system of the airways have helped suggest rationale therapies for keeping CF lungs healthy.

Congratulations to these extraordinary CF community heroes!

Cystic Fibrosis Research Inc.

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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

CFRI's "Breathe In ~ Work Out" CF Wellness Initiative

CFRI's "Breathe In – Work Out" CF Wellness Initiative was 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.

Classes Include:

Yoga for CF Community Wellness

Physical Therapy for CF Health

Pound for CF Health – Music, Rhythm & Health

CF Knowledge in Motion Exercise Program

For more information and upcoming class dates, please check our website at **www.cfri.org**

Sponsored by a Vertex Pharmaceuticals Circle of Care Grant.

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