

CF Advocacy: The Personal is Political

By Jacob Fraker

I first became aware of advocacy when I was 7 or 8 years old. As a child with cystic fibrosis (CF), I was in the hospital for a procedure which involved threading a tube with a camera up my nose and down to my stomach. My parents had given permission, but I was having none of it. I'd been in the hospital for a week already and had been poked and prodded with multiple blood draws, a PICC line insertion, an MRI, and I had reached my limit. I was fighting back with all my might as the nurses and techs held me down while they tried to push the tube up my nose. Then my mom came in the room and said firmly, "Enough. We are not doing this today." I was a child and had no say, yet the people I trusted and loved were able to step in and say on my behalf, "enough." It was life changing.

Advocacy comes in many forms and isn't always at a political level. It can happen person to person or within your community. I am happy to serve as CFRI's legislative and governmental analyst, and to connect others with CFRI's Many Voices ~ One Voice CF Advocacy and Awareness Campaign. In doing so, I hope to bring your stories and your voices to state and federal legislators. Together we are a powerful community.

My commitment to advocacy increased while working at nonprofit organizations, where I always aspired to get to the root of the problem in addressing a community need. My first opportunity to work at the state level came when I was selected for a fellowship

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CFRI advocates in Sacramento for declaration of CF Awareness Month in CA

Assessing the Role of Secretory and Absorptive Epithelium Lining Conductive Airways



Paul Quinton, PhD & Guillermo Flores-Delgado, PhD

By Guillermo Flores-Delgado PhD, and Paul Quinton PhD, Pediatrics, UC San Diego

A thin layer of airway surface liquid, thinner than a hair, is crucial to protect the surface of the inside of bronchi and bronchioles from the accumulation of toxic material and debris trapped in mucus. Abnormalities in this airway surface liquid (ASL) can lead to fatal consequences. Therefore, it is imperative to understand how this thin layer is produced and maintained to be able to design better therapies for airway disease.

In the upper large airways, submucosal glands beneath the airway lining epithelium are the main source of liquid secretion, which is largely reabsorbed by the cells of the surface epithelium. However, in the small airways of the more distal regions of the lung, there are no submucosal glands, and how airway surface liquid is produced remains poorly understood.

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Winter 2017

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Letter from the Executive Director

Dear Friends,

As the year draws to a close, I reflect on the tremendous progress in CF research and therapies for those with cystic fibrosis (CF), as well as CFRI's commitment to meeting our CF community's diverse needs. While we celebrate that the median life expectancy for a person born with CF is now 47, quite tragically, last year half the individuals who died from CF were under the age of 30. We still have work to do, and together we can accomplish great things.

I thank all of you who joined our Many Voices ~ One Voice CF Advocacy and Awareness Campaign this year. Thus far in 2017, CFRI implemented over 40 calls to action to our 17,000 constituents. Thousands of emails were sent to federal and state decision makers, addressing such topics as: the OPEN ACT; NIH funding; attacks on the Affordable Care Act; the 21st Century Cures Act; and expedited FDA approval of drugs. Thanks to you, our engagement rates averaged 13.68%, far above the industry standard of 1-3%. It takes many voices to be heard – and our legislators heard you loud and clear!

CFRI is your partner in living, and in 2018 we hope to expand our online programs and national counseling program. Our in-person group events, including the National CF Family Education Conference, Mothers Retreat, CF Summer Retreat, and podcast events will continue, offering community members the opportunity to come together to learn and to share.

Thank you for your continued support of CFRI's CF research, education, psychosocial support, and advocacy programs. Thank you for your support of each other. I encourage you to donate to one of our year-end giving campaigns. Together we can hold up the sky.

Warmly,



Sue Landgraf | CFRI Executive Director and Mother of an Adult Daughter with CF



Sue Landgraf, Executive Director

News from the Board

Dear CFRI Community,

I am honored to serve as President of CFRI's Board of Directors, and am inspired by the efforts of many who channel their energies and resources into prolonging and enhancing the lives of those with CF. Our continued growth and program expansion is thanks to you – the members of our dynamic community.

CFRI has had another successful year. Financially we are on solid ground. Our funding is derived from a wide range of sources, including corporate and private foundation grants, Mothers Day Tea, gala, workplace giving, marketing contracts, and private donations. People across the country are "donating" their birthday to CFRI via Facebook, while our Reaching New Heights Campaign inspired "Cocktails for a Cure," and a fun Zumba event. Thanks to the many ways that our community supports us, we will end 2017 with purpose and inspiration.

2018 will bring more growth. We want your input and your involvement. I thank you for your steadfast support and hope you will join me in helping CFRI to reach even greater heights in 2018.

Peace and good health,



Bill Hult | President, CFRI Board of Directors



Bill Hult, President

CF Advocacy: The Personal is Political

Continued from front cover

with Equality California, an LGBTQ rights group, to work with a state senator on LGBTQ equality and other issues he was involved with. I got to see where the political rubber meets the road, and to advocate for those with rare diseases, for the LGBTQ community and beyond. It was an incredible opportunity to work with underserved groups to figure out their issues and needs, and convey their concerns to people in a position to make a difference.

Some people hesitate to become an advocate, worrying that they have no power or influence. It is important to remember that elected officials work for us, not the other way around, and that they are aware their jobs are up for renewal every few years. During my fellowship I saw many examples in which public input – including a flood of calls and emails – led to support of a bill which then moved through committee. There is a misperception for many who say, “Why call? I’m only one voice,” but when we come together, we can become a strong movement.

The “rare disease” and CF communities are large and diverse and our issues may intersect in ways initially unseen, but when

identified can greatly increase our influence to affect change. For example, there was once a bill proposed in California addressing surrogacy. Of course this issue not only impacts men and women with CF, but also impacts the LGBTQ community. If your issue is not explicitly CF-related, then it is important to talk about it and explore cross impacts and networks.

I enjoy working in Sacramento on CFRI’s and the CF community’s behalf to ensure that those in office are held accountable. It can be difficult sometimes, and of course there are wins and losses. That is what advocacy is about – speaking out and finding new ways to have our stories heard.

I feel very fortunate to be able to work with CFRI in this capacity. I know that for many with CF, it may not be an option for a wide variety of reasons. But CFRI and I want to hear from you and share your



Jacob Fraker with California State Senator Henry Stern

stories. We are now evaluating legislation to determine its benefits or risks for those with CF, expanding our work with the California Rare Disease Caucus, and will likely propose legislation to increase our community’s access to therapies. I hope you will join us and be a part of our efforts. You deserve to be heard and respected. Let’s accomplish something together.

To participate in CFRI’s Many Voices ~ One Voice Campaign, please email cfri@cfri.org.



“Those who are happiest are those who do the most for others.”

— Booker T. Washington

When 21-year-old Jessica Fredrick lost her battle with cystic fibrosis (CF), her family sought to generate hope out of tragic loss.

Doing the Most for Others: The Jessica Fredrick Memorial CF Research Challenge Circle and Fund

Thanks to the generosity of her aunt, CFRI’s Jessica Fredrick Memorial CF Research Challenge Circle and Fund were born. Circle members give generously to create a pool of funds used to match gifts for our research awards and to inspire others to join the search for new CF therapies and a cure.

This year, members of our Circle contributed over \$85,000 and matched donations – dollar for dollar – from individuals committed to CF research. Together with donations to the Jessica Fredrick Memorial CF Challenge Fund, over \$170,000 was designated for our CF research awards.

Please join this inspiring group! Become a member of the 2018 Jessica Fredrick Memorial CF Research Challenge Circle by

making a minimum gift of \$2,500. You can make your gift in 2017, and your contribution will be restricted to our research award program for future New Horizons and Elizabeth Nash Memorial Fellowship awards.

If you are unable to join the Circle, please consider making a year-end gift to the CF Research Challenge Fund, which will be designated for CF research awards. Whichever you choose, you will inspire our community to do the most for others to make the dream of a CF cure a reality.

Despite tremendous progress in CF therapies, we continue to lose our loved ones to this cruel disease, and there is still no cure. We need your help to improve and save lives. Please give generously today.

Assessing the Role

Continued from front cover

Using electrophysiological methods, our laboratory discovered that small airways are capable of both fluid secretion and absorption that maintain the surface liquid, but the source of cells in the small airways was not defined. We used the expression of NKCC1, a transport molecule essential for fluid secretion to identify specific cells with secretory function. We discovered that in distal airways, cells with NKCC1 were distributed in regions in the furrows of the pleated surfaces of small airways. Whereas in large airways, secretory cells were mainly in the submucosal glands, not in the luminal surface epithelium.

On the other hand, the epithelium sodium channel (ENaC) is an excellent marker consisting of α -, β - and γ - subunits in absorbing epithelial cells, so we used antibodies to detect cells with these subunits of ENaC. Our histochemical analysis demonstrated that the three ENaC subunits appeared mainly in the ciliated cells of the airway

surface epithelia. In distal small airways, α - and γ - ENaC subunits were present at the base of the cilia. β -ENaC subunits were distributed more narrowly in ciliated cells in ridges of the epithelial pleats, where it was prominent along the cilia. In the large bronchial airways, ENaC subunits were also present mainly in ciliated cells all along the luminal surface epithelium, but were absent in the epithelia of submucosal glands.

In summary, we demonstrated that distal and proximal airways contain different types of cells that either secrete or absorb depending upon where they are located in the surface epithelium as recognized by the presence of either NKCC1 or ENaC subunits in them, respectively. In distal small airways, the distinct expression of NKCC1 or the β -ENaC subunit indicates that the cells of the epithelium are segregated into uniquely secretory or absorptive cells that maintain the ASL by simultaneously reabsorbing fluid on the airway surface. In large proximal airways, the expression of ENaC subunits indicates that the surface



Guillermo Flores-Delgado, PhD

epithelium is dedicated to absorption, while the expression of NKCC1 indicates that submucosal glands are dedicated to secretion. Our findings provide novel insights into how distinctive cells are distributed in the airways to produce and maintain ASL, which may aid in the developing cell-specific therapies in CF and other airway diseases.

Study on CFRI's Counseling Program Finds Significant Impact Upon Participants

By Siri Vaeth, MSW and Rick Moss, MD

We know from The International Depression Epidemiological Study (TIDES, Quittner A, et al, 2014) that members of the CF community have rates of anxiety and depression two to three times greater than community samples.

Psychotherapy and individual counseling support have shown to decrease anxiety and depression, and improve coping skills and quality of life. There are many issues preventing access to mental health services,



among which financial barriers are significant. It is critical to address mental health, because of its direct impact upon adherence to medical regimen, quality of life and health outcomes.

In 2015, CFRI was awarded a Circle of Care Award from Vertex Pharmaceuticals

to initiate a program through which CFRI provides funding for five sessions of individual therapy to those with CF and their family members with a licensed provider of their choice. As recently presented at the North American Cystic Fibrosis Conference, a study to evaluate the impact of these sessions among participating individuals with CF and/or their family members demonstrated that the program is very successful in reducing symptoms of depression and anxiety.

In 2016, 53 individuals initiated therapy; by year-end, 43 from 11 states had completed five sessions along with pre and post-therapy Generalized Anxiety Disorder 7-Item (GAD-7) and Patient Health Questionnaires (PHQ-9). Of these, 74% were women and 60% were individuals with CF.

Pre-therapy, participants' mean GAD-7 score was 9.77, indicating mild to moderate anxiety. Post-therapy, the mean dropped to 5.77 – a statistically significant improvement. The pre-therapy PHQ-9 mean score for the group was 9.52 (mild to moderate depression); after five sessions the mean had a statistically significant drop to 5.77.

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cfri | Winter 2017

Save the Dates!

CF Caregivers Support Group

(Attend in Person or by Phone):

Dec. 19, Jan 16, Feb. 20

CF Caregivers of Children
5:00 pm – 6:00 pm PST

CF Caregivers of Adults
6:00 pm – 7:00 pm

For location or call-in number,
go to www.cfri.org

Introduction to Mindfulness Based Stress Reduction Online Class for Those with CF & CF Caregivers

Taught by Julie Desch, MD
February dates announced soon
6:00 pm – 7:30 pm PST

Embrace: CFRI Mothers' Retreat

May 4, 2018 – May 6, 2018
Vallombrosa Center
Menlo Park, CA

Sponsored to date by
Vertex and Gilead Sciences

31st National CF Family Education Conference

Bridges to the Future
August 3 – August 5, 2018
Pullman SF Bay
Redwood City, CA

Sponsored to date by
Vertex and Gilead Sciences

CFRI's CF Summer Retreat

August 16 – August 21, 2018
Vallombrosa Retreat Center
Menlo Park, CA

Sponsored to date by Gilead Sciences

Enchanting Adventure at Nestldown

September 29, 2018
Nestldown
Los Gatos, CA

Sponsored to date by
Vertex Pharmaceuticals

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

In Honor of

September 1, 2017 — October 31, 2017

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Sadie Anderson
Jessica Arvidson
Alex Avant
Bridget and John

Barnes
Lucy Barnes
Lucy, Charlie and Dylan
Barnes Markell

Brandon Begin
Brett Bennett
Ryan Bortz
Brian Burks
Sean Campise
Lauren Colonna
Cameron Cornell
Barbara Curry

Barbara and Jim Curry
Tess A. Dunn
Debbie Duplessis
Daniel Ellett
Hayden Ellett

Timothy Estabrook
Kristin Favero
Konvolinka
Victoria Flamenco

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Robby and Jay
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Schultz

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Thompson
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Kassi Watkins
Jeff Wine, PhD

In Memory of

September 1, 2017 — October 31, 2017

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Ann Maureen and
Ronald Baldwin
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Irvin Beltrame
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Jane Ellen Kulik

Jim Landis
Alan Leventhal
Matthew Lewis
Robert Mackey
Marian's grandson
Richard Marias
Nahara Mau
Merlin Maynard
David McAfee
Jerome Medowar
Nardya Miller
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Edna Rogers
Thelma Rosenthal
Dhea Schalles
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Steven L. Shepherd
Tammy Smerber
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Thibault
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Hayley Wester

Tributes

Our "In Memory of" and "In Honor of" pages provide the opportunity to honor a person, family, or special event, 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 acknowledgement of your gift to the person you designate.

Please mail your contributions to:

CFRI 1731 Embarcadero Road, Suite 210, Palo Alto, CA 94303



CFRI and Me: Together We Are Partners for Life

There are Many Easy Ways to Deepen Your Relationship

- **VOLUNTEER** in the CFRI office, or at a CFRI event. Serve as an Ambassador, or lend your IT skills. The sky's the limit as to what's possible. If you have time, desire and ideas, let us know and we'll help you make it work.
- **ATTEND a CFRI FUNDRAISING EVENT**, or hold your own! Check out our listings at cfri.org, or we'll help you through our *Reaching New Heights* Campaign.
- **TRIBUTES: In Honor Of and In Memory Of**. Recognize a loved one with your choice of gift. CFRI will then send an acknowledgement letter to your designee.
- **GIVE STOCK to CFRI**. Donating appreciated stock avoids paying capital gains taxes 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** potentially offers increased income, tax savings, the opportunity to meet your philanthropic goals, and the satisfaction of making a 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.
- **VEHICLE DONATIONS**: Donate your car/RV/boat to CFRI. Your contribution is tax-deductible, and we will coordinate the transfer of property.

For more information, please contact **Tony Adessa, CFRI's Development Manager**: 650.665.7586 or tadessa@cfri.org.

Study on CFRI's Counseling Program

Continued from page 4

CFRI's results were consistent with TIDES, as CF family members had the highest rates of depression and anxiety pre-therapy. The reduction in their symptoms post-therapy was statistically significant (Pre-PHQ-9=11.12 / Post=5.65; PreGAD-7=10.41 / Post=6.06). Individuals with CF had a pre-therapy PHQ-9 mean score of 8.12; post-therapy mean was 5.46. The group's pre-therapy GAD-7 mean was 9.00; post therapy was 6.62 ($p < 0.037$).

Cystic fibrosis co-morbidities have a significantly negative impact on patient and family members' mental health and quality of life. Rapidly declining health, fear for one's child, financial hardship, marital stress after a child's diagnosis – these issues are prevalent in our community. Short-term individual therapy successfully reduces symptoms of depression and anxiety found among those impacted by CF, thereby improving quality of life.

Program provided via funding awards from Vertex Pharmaceuticals and Genentech, as well as the CF Quality of Life Program – A Living Legacy of Peter and Kathy Judge.

Raising CF Awareness Takes a Village

Watch and share CFRI's 2017 Many Voices ~ One Voice CF Advocacy and Awareness video. Featuring members of our CF community, this moving film shares facts about cystic fibrosis and seeks to inspire viewers to join CFRI's advocacy campaign. Please share the link with your friends, family and colleagues! To watch, go to <https://tinyurl.com/ybkwzbtm>.



CFRI's Moonlight Masquerade a Thrilling Success!

CFRI's Moonlight Masquerade exceeded all expectations! Guests enjoyed the beautiful Hillsborough Racquet Club, along with gourmet food, wines, a very enthusiastic auction, live music, and late night dancing with a DJ. CFRI expresses warm appreciation to Jim and Barbara Curry, who generously co-hosted the event. In addition to the festivities, guests were inspired by Alisa and Randy Evans' moving story of their son Tommy's journey to transplant. We proudly honored Dr. Rick Moss with CFRI's 2017 CF Champion Award, in recognition of his many contributions to cystic fibrosis research and clinical care, as well as to the CF community. Thanks to the support of many, the gala net over \$113,000 for CFRI's research, education, advocacy, and psychosocial support programs.

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

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The Sea at Alexander's

Steakhouse

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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.

Visit our website at:

www.cfri.org

for more information about us and about cystic fibrosis.

Call toll free: 855.cfri.now

Reaching New Heights – Supporting CF Solutions

*Host a fundraiser in your community!
Help those with cystic fibrosis while having fun, raising CF awareness and changing lives.*



The ideas are endless and CFRI will help you to succeed. We would love to recognize you on Facebook and Instagram, and in our weekly eNewsletter.

- Neighborhood Yard Sale
- Cocktails for a Cure
- Golf Tournament
- Gift Wrap Table
- Holiday Arts & Crafts Boutique
- Musical or Theatre Performance
- Walk-a-thon / Cross Fit / Jog-a-thon
- Pet Fashion Show
- Whimsical Tea Party
- Silent Auction

Let's Reach New Heights & Find Solutions for Those with CF!

Contact Tony Adessa at CFRI to discuss ideas and how CFRI can support you!
650.665.7586 / tadessa@cfri.org

For their generous support of **CFRI Community**, special thanks to:
Genentech, Vertex Pharmaceuticals, Gilead Sciences, AbbVie & Chiesi USA
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