New CF Drugs Change the Face and Future of Cystic Fibrosis

By Bridget Barnes

With the recent approval of new drugs, hope is the driving force in the future of cystic fibrosis (CF). In her engaging update, “The CF Therapeutic Pipeline: Ushering in an Era of Great Hope,” Dr. Margaret Rosenfeld, Professor of Pediatrics at the University of Washington and attending pediatric pulmonologist for 25 years at Seattle Children’s Hospital, greeted CFRI’s 28th National CF Family Education Conference audience saying, “We are all partners in progress and can work together to achieve our goal that all persons with cystic fibrosis will live full, healthy lives.”

Dr. Rosenfeld reviewed basic CF pathophysiology, describing how the faulty CFTR gene leads to defective chloride transport, causing respiratory airway surface liquid depletion, impaired mucociliary clearance, mucus obstruction, and ultimately, inflammation and infection of the airways. Identifying the therapeutics pipeline as, “a whole cascade of drugs,” her primary focus was on CFTR potentiating and correcting modulators, Kalydeco and lumacaftor.

Targeting the basic defect in cystic fibrosis, “the new CFTR modulators are responsible for this era of hope,” Dr. Rosenfeld explained. Kalydeco is now approved for two to five year olds with gating mutations, thus allowing initiation of CFTR modulation before structural airway damage can occur. In July, the lumacaftor/ivacaftor combination drug Orkambi was approved by the U.S. Food and Drug Administration (FDA) for people 12 years and older having two copies of F508del, potentially benefitting nearly one-third of the people living with CF in the U.S. Before taking Orkambi, Dr. Rosenfeld recommended working with one’s pharmacist to discuss possible side effects and interactions with other CF drugs.

Dr. Rosenfeld noted that CF genetic mutations fall into one of five mutation classes. To restore defective

Correcting Abnormal Cystic Fibrosis Mucus with CFTR-independent Bicarbonate (HCO3-) Secretion

By Ning Yang, MD, PhD

Although viscous, adherent mucus is pivotal in the development of cystic fibrosis (CF) pathology, how mutations in the CFTR gene lead to pathogenic mucus accumulation and how to correct the abnormally thick mucus is only beginning to be understood. Our recent studies showed that normal mucus release requires concurrent CFTR-dependent bicarbonate (HCO3-) secretion in CF-affected tissues such as mouse small intestine, the female reproductive system, and a human lung cell line. HCO3- appears to function as a calcium (Ca2+) chelator to help disperse secreted mucin granules (Garcia et al., 2009; Chen et al., 2010; Muchekehu & Quinton, 2010; Quinton, 2010: Yang et al., 2013). In this CFRI-funded project, we further investigated the role of CFTR-dependent HCO3- secretion in the regulation of human cervical mucus, and tested our hypothesis.
Letter from the Executive Director

Dear Friends,

What a whirlwind of activity accompanied these past few months! Our 28th annual National Cystic Fibrosis Family Education Conference brought together 200 members of our community from as close as Palo Alto and as far away as Australia to learn about the latest in cystic fibrosis (CF) therapies and topics that are vital to those living with the disease. The recent approval of Vertex’s new drug Orkambi will help many, while there are numerous promising CF therapies currently in the pipeline. It is truly a time of hope.

As CFRI marks its 40th year, we celebrate the progress that has been achieved, while recognizing the challenges that remain ahead. A cure for CF still eludes us, and for many with advanced lung disease, new therapies may not arrive in time. CFRI is committed to meeting the multi-faceted needs of our community, including our educational programs (in English and Spanish), which help those with CF and their loved ones to become educated advocates for optimal health care, as well as our programs that address the mental health issues associated with CF, and help members of our community to improve their quality of life.

We remain strongly committed to CF research, and have recently revised our research award programs. In 2016, we will expand our national reach for both the Elizabeth Nash Memorial Fellowship and New Horizons Research programs, while awarding larger grants along with two-year project commitments. We seek to invest in the highest quality scientific research that will broaden treatment options, improve quality of life, and expand the search for a cure.

Of course, when I say “we,” I am including you. Those of you who support CFRI are partners in both past and future discoveries. I encourage you to contribute to the Jessica Fredrick Memorial CF Research Challenge Fund. All gifts made through this newsletter will be generously matched by the Research Challenge Circle. We can sustain our expanded research with your help.

I am the mother of an adult daughter with CF, and have recently experienced both the heartbreak and triumphs that accompany life with CF. I am honored to be a part of this caring community.

Warmly,

Sue Landgraf  
Executive Director and mother of an adult daughter with CF

News from the Board

Dear CFRI Community,

I am delighted to share that CFRI’s Board of Directors has grown to include two dedicated experts in the field of cystic fibrosis (CF): Rick Moss, MD, and Ronni Wetmore, RN, MS. Rick is the former chief of the Stanford Pediatric Pulmonary and Allergy Divisions and was director of the CF Center at Stanford from 1991 to 2009. He helped to launch the CF Quality of Life (CFQol) Program and serves on CFRI’s CFQol Advisory Board. Ronni is the Nurse Coordinator at the Adult CF Center at Stanford. In addition to her extensive professional nursing career,
What’s New in Infection Prevention and Control?

By Eric Frisbee

CFRI was honored to welcome renowned epidemiologist Lisa Saiman, MD, MPH, to its National Cystic Fibrosis (CF) Family Education Conference. A professor of pediatrics at Columbia University Medical Center and an attending physician at New York-Presbyterian Morgan Stanley Children’s Hospital, Dr. Saiman’s medical research and clinical interests involve the infectious disease and microbiology found in those with CF, with an emphasis on multidrug-resistant pathogens.

Dr. Saiman’s experience includes infection prevention and control in the neonatal ICU, and with critical pediatric patients recovering from bone marrow transplants. CF infection prevention protocols within the healthcare facilities will differ from practices in everyday life. Dr. Saiman states, “The challenge with cystic fibrosis infection prevention and control is to understand the risk versus benefit so people do not live in a bubble.”

According to Dr. Saiman, the hospital and CF clinic are high-risk facilities for those with CF. Minimizing the time that more than one person with CF is in a clinic waiting room is a difficult but helpful tool in reducing cross infection risks. The transfer of pathogens might occur through tests commonly performed in clinic, including increased exposure to respiratory secretions and saliva created during pulmonary function tests, sputum sample collection, and respiratory therapy. Any cough-inducing trigger increases the incidence of respiratory droplets. The goal in the CF clinic is to prevent cross infection between people with CF who must be seen under one roof in a relatively small space.

The benefit of routine CF clinic visits is well established, with the risk of infection transfer between patients mitigated through recommendations in the Cystic Fibrosis Foundation’s (CFF) CF Infection Prevention and Control (IP&C) guidelines, which in 2013 underwent their first revision in a decade. In revisiting the infection control recommendations for patients with CF microbiology, and infection control practices to prevent patient-to-patient transmission, several data-driven changes were made. The 2013 guidelines recommend that pulmonary function tests (PFT) be performed in an exam room at the beginning of a clinic visit using portable instrumentation, in a PFT lab under negative pressure, or in a PFT lab with high-efficiency particulate arrestor (HEPA) filtration. Depending on the resources of the clinic, an alternative option is to allow the air in the PFT room to settle or clear for 30 minutes between patients.

Nebulizer sanitation can be tricky and it is recommended that patients work with their CF care team to optimize sterilization specific to each nebulizer. Vinegar (acetic acid), bleach solutions, and benzalkonium chloride are no longer recommended for decontaminating nebulizers because non-tuberculous mycobacteria is very hardy, and may be resistant to bleach or vinegar. When possible, nebulizer decontamination by heat is very effective: submerge the nebulizer in boiling water for 5 minutes, or microwave non-metal nebulizers submerged in water for 5 minutes.

Pathogens spread through both direct and indirect contact. An example of direct contact is hand shaking, while indirect spread may occur from a cough creating respiratory droplets. Covering the mouth when coughing, and hand hygiene after coughing, can effectively control respiratory droplets. The majority of all bacteria, viruses, and molds are found everywhere in the environment, with high concentrations in soil and stagnant water. The most important infection control practice against all pathogens is hand hygiene, which applies to everyone, from healthcare personnel and those with CF, to the entire community.

For people with CF, balancing infection prevention and control with quality of life is essential to preserving wellbeing and self esteem. With awareness of infection prevention backed by solid information, those impacted by CF can weigh risks and benefits and maintain self-agency while making sound decisions so as to preserve this vital balance.

News from the Board
Continued from page 2

Ronni has a deeply personal connection to CF, having lost two siblings to the disease. Our new directors’ expertise and personal experience will have a vital impact upon CFRI.

I am also honored to introduce the newest member of our Medical Advisory Board, Lisa Saiman, MD, MPH. Lisa is a Professor of Pediatrics at Columbia University Medical Center, an Attending Physician and Hospital Epidemiologist at New York-Presbyterian Morgan Stanley Children’s Hospital, and a recognized expert in the epidemiology of the infectious complications of CF. Welcome Lisa!

CFRI wishes to thank Marybeth Howard, PhD, for her 12 years of service as CFRI’s Research Advisory Committee (RAC) Chair, overseeing CFRI-funded quality research projects. Marybeth will remain on the committee, and I am delighted to welcome Julie Desch, MD, as the new RAC Chair. Julie has been a member of the RAC for years, and we are fortunate that she has accepted this leadership role. I also wish to recognize Beate Illek, PhD, director of CFRI’s Elizabeth Nash Fellowship Program, which now has a national reach; her leadership will play a key role in this exciting expansion.

Peace and good health,

Bill Hult | Board President
New CF Drugs Change the Face & Future of CF
Continued from front cover

CFTR function, there are two types of modulators. “Correctors” (lumacaftor) help molecules get CFTR to the surface of the cell for people with Class I, II or III mutations. “Potentiators” (Kalydeco) help molecules on the cell surface function properly for those with Class IV or V. Kalydeco, which helps 4% of those with CF, “was a breathtaking and audacious advance in CF treatments,” Dr. Rosenfeld explained, improving lung function and weight gain, dropping sweat chloride levels, reducing hospitalizations, and decreasing Pseudomonas infections.

In people with one copy of F508del, the clinical response to Orkambi was insignificant. Vertex Pharmaceuticals, creator of Kalydeco and Orkambi, has additional therapies in development and is conducting clinical trials of Kalydeco combined with VX-661, with people carrying either one or two copies of F508del. Other studies indicate that adding a second corrector to a potentiator further enhances in vitro F508del CFTR function.

Correcting Abnormal CF Mucus
Continued from front cover

that pharmacological management of CFTR-independent HCO3- secretion can ameliorate abnormal mucus in cystic fibrosis.

With improved health status, higher survivability, and reduced disease-associated morbidity, the number of pregnancies in women with CF appears to be increasing (Canny et al., 1991). Nonetheless, the conception rate of women with CF remains significantly depressed (Sawyer, 1995). In this project, we further tested altered pH and cervical mucus in the reproductive tract of women with CF. The mean baseline (day 21-30) cervical pH in CF patients was not significantly different from that in controls. However, the pH of mucus in controls during the ovulatory period increased 0.48 pH units, while the cervical pH in CF patients only increased 0.29 pH units, indicating that more HCO3- was secreted in the reproductive tract in healthy women controls than in CF. On the other hand, the viscosity of control mucus samples significantly decreased about 14 fold during ovulation while the mean viscosity of CF specimens during ovulation decreased only about 3 fold, suggesting that the mucus thinning that normally accompanies the increase in pH in mucus secretions from control subjects during ovulation was absent in CF women. These observations link the basic defect in CFTR-dependent anion transport to the mucus pathology seen in CF and implicate poor HCO3- secretion as a significant factor in the subfertility found in women with CF. Lubiprostone, an FDA approved drug for the treatment of chronic constipation, has been shown to ameliorate the CF mouse intestinal phenotype (De Lisle, Mueller et al. 2010). In testing with lubiprostone, we found that it induced significant HCO3- secretion which was not completely inhibited by the CFTR inhibitor, GlyH-101. These results suggest that lubiprostone induces CFTR-independent HCO3- secretion in mouse ileum.

We then examined the effects of lubiprostone on mucus release. In our previous study, potent mucus secretagogues (serotonin and prostaglandin E2) stimulated mucus release, which was almost completely blocked by GlyH-101 (Garcia et al., 2009). In contrast, in the presence of HCO3-, lubiprostone still stimulated significant mucus release even with GlyH-101 present. Likewise, in the presence of GlyH-101, lubiprostone-stimulated mucus release was greatly reduced by removing HCO3- from the bath solution, indicating that lubiprostone-induced CFTR-independent HCO3- secretion can restore abnormal mucus release caused by defective CFTR.

Our results suggest that the mucus thinning that normally accompanies the increase in pH in mucus secretions from control subjects during ovulation was absent in CF women, indicating poor HCO3- secretion as a significant factor in the subfertility found in women with CF. CFTR-independent HCO3- secretion may correct CFTR dependent mucus release. This study provides new insights into treating and alleviating pathogenic mucus in CF.

This research was funded in part through a CFRI New Horizons Research Grant. GlyH-101 is a generous gift from Dr. Alan S. Verkman, UCSF.
The Birds, The Bees and Cystic Fibrosis

By Siri Vaeth Dunn, MSW

Anna Tsang, MSN NP, CDE, jokes that she earned the title of “sex queen” while serving as the nurse coordinator at the Adult CF Clinic at St. Michael’s Hospital in Toronto, Canada from 1994 to 2014. During her enlightening presentation, “The Birds & the Bees: CF and Reproductive Health,” at CFRI’s 28th National CF Family Education Conference, Tsang spoke compellingly about the need for improved education and communication to address sexual and reproductive health issues facing teens and adults with cystic fibrosis (CF).

Tsang recommends that CF teams communicate clearly with their patients about the impact of CF upon reproductive health. Many with CF experience delayed puberty; boys may have smaller stature, while girls may have later onset of menarche than their peers. Adolescence is a time of intense body awareness, and the team should be aware that many teens have negative self-image due to G-tubes and porta-caths.

Over 98% of men with CF are infertile due to bilateral absence of the vas deferens (CBAVD) and this issue must be addressed early on. Tsang stressed that, “infertility does not equal impotence, and sexual function is not affected.” Men with certain CFTR mutations are fertile, and Tsang believes pediatric clinics should offer semen analysis to young men in their late teens. For those with CBAVD, healthcare teams must stress that while pregnancy is not an issue, protection must be used to prevent sexually transmitted diseases.

Young women with CF need to be aware that antibiotics frequently cause yeast infections, while urinary incontinence is common due to intense coughing. Tsang noted that the care team must raise these issues with their patients, who may be embarrassed and suffering unnecessarily.

While thick cervical mucus can impact fertility for women with CF, the belief that women with CF will not become pregnant is “a myth.” Contraceptive options should be discussed, including oral contraceptives, IUDs and vaginal rings. Tsang noted that Depo-Provera should be avoided due to its effect on bone mineral density.

Cystic Fibrosis and Mental Health

By Isabel Stenzel Byrnes, MSW, MPH

Attendees at CFRI’s 28th National CF Family Education Conference had the honor of welcoming two cystic fibrosis (CF) social workers, Kimberly Morse, LCSW, from Children’s Hospital Los Angeles, and Martha Markovitz, LCSW, from University of Southern California’s Keck Medical Center, to discuss the critical link between mental health and medical adherence in children and adults with cystic fibrosis.

Morse and Markovitz provided an excellent summary of mental health issues impacting cystic fibrosis patients, including symptoms of depression and anxiety in both children and adolescents. Because of developmental differences, these age groups present with different symptoms than adults. Both Morse and Markovitz acknowledged that many CF patients suffer from fatigue, changes in sleep and appetite, and struggle with feelings of irritability and lack of motivation because of the physical aspects of the disease. So how does one separate CF from CF-related depression and/or anxiety? Depression and anxiety can spiral and negatively impact physical health, and simply acknowledging the relationship between mind and body can help patients understand their unique situation.

Morse and Markovitz reviewed research indicating that depression is highly associated with poor medical adherence and unfavorable outcomes in CF. Interestingly enough, researchers have found that anxiety is more common than depression for those with CF, but there is a positive relationship between anxiety and compliance. While anxiety can be adaptive because it motivates us to pay attention, in the long term it can lead to sleep disturbances, somatic complaints and other problems. Markovitz suggests we deconstruct anxiety to pull it apart, examine it, and identify strengths and abilities to overcome fear, to cultivate hope, and to develop resilience.
For those with CF wishing to start families, Tsang emphasized the importance of a comprehensive education and counseling session with the CF team. Options include adoption, artificial insemination and the use of artificial reproductive technologies (ART). Regardless of the steps to parenthood, Tsang noted that prior to making the decision, there must be a frank discussion about the possibility of the early death of the CF parent.

Tsang stressed that ideally women with CF should plan their pregnancies, as the CF team will work with the obstetrician, endocrinologist and other specialists. Women with good nutrition and stable lung function (FEV1 of 50% or greater) have better outcomes. Tsang cited several studies demonstrating that pregnancy does not shorten survival, and that maternal and fetal outcomes are usually good. She added, “Experience shows that it is motherhood – not pregnancy – that may lead to a decline in one’s health.” With progress in transplantation, pregnancy post-lung transplant is possible, but the risk of organ rejection is increased with pregnancy. Four of the five most commonly prescribed immunosuppression drugs show evidence of fetal risk. It is suggested that women wait at least two years post transplant for more favorable outcomes.

Sexual health and reproductive education should be an integral part of CF care, delivered in a well planned, developmentally appropriate manner which involves parents and both pediatric and adult CF teams. In closing, Tsang encouraged each CF clinic to find their own “sex queen or king” to take the lead in addressing these issues.

Morse and Markovitz reviewed the ongoing TIDES Study (The International Depression Epidemiologic Study) of 10,000 CF patients in Europe and North America, which found that CF patients suffer from depression at two to three times the rate of the mainstream population. Approximately 5% – 19% of teens, and 13% – 19% of adults with CF struggle with depression, while 22% of teens and 32% of adults struggle with anxiety. Markovitz and Morse emphasized that most patients with CF (70% – 80%) do not have anxiety and depression, and have significant strengths to cope with their CF-related adversity. Notably, 37% of mothers and 31% of fathers in this study also struggle with depression, while 48% of mothers and 36% of fathers struggle with anxiety. This highlights the need for parents of children with CF to receive effective mental health intervention, especially since parental coping can significantly impact a child’s ability to cope with CF.

Due to the TIDES study, led by Alexandra Quittner, PhD, the Cystic Fibrosis Foundation implemented mental health guidelines in 2013. Screening is encouraged to differentiate “normal” reactions to loss or “normal” rebellion, and non-adherence due to depression and anxiety.

Morse reviewed the evidence-based treatments available for patients with depression and anxiety, including different modalities of counseling and psychotherapy, as well as medications. A collaborative care model involving the patient, CF team, psychiatrist/psychologist, and pharmacist is required for effective mental health care. Morse and Markovitz also encouraged online support for CF patients, family centered care, goal setting, and working on positive cognitive appraisal, which includes hope and positive self-esteem.

As a social worker and person living with CF, I have long believed that CF is both a physical and emotional illness. It is encouraging that CF centers are paying closer attention to the impact that mental health has upon physical health. Morse and Markovitz effectively highlighted the capacity for resilience that exists in those living with CF.
Living a Full Life with CF: How the Law Can Help
By Siri Vaeth Dunn, MSW

CFRI was honored to welcome Beth Sufian, JD, as a returning presenter to our 28th National CF Family Education Conference. As a 49-year-old attorney with cystic fibrosis (CF), Sufian is driven to help others with CF understand the laws that protect them. Since 1998 she has been the director of the CF Legal Information Hotline, which has received over 40,000 calls. Sufian is also director of the CF Social Security Project, which has represented and successfully obtained Social Security benefits for over 1,000 individuals.

“The landscape of social security has entirely changed,” noted Sufian. Social Security Insurance (SSI) benefits provide income support and eligibility for Medicaid and Medicare to children and adults with CF. The system is under increasing pressure from Congress to lower costs, causing many with CF to have their SSI benefits reviewed. In many states adults must receive SSI to be eligible for Medicaid and the disruption of benefits can be catastrophic.

Medical criteria for SSI and Social Security Disability Insurance (SSDI) include one of the following: low FEV1; three hospitalizations in the past year; six physician interventions; or hemoptysis. Other criteria include the use of home IVs or inhaled antibiotics every six months for pulmonary infections, plus significant daily time dedicated to respiratory therapy. Even if one does not go to clinic for these issues it is imperative to call and have a note included in the official health record.

Once individuals with CF turn 18, parental income is not included in an SSI review, but they must meet SSI medical criteria and cannot engage in full time work. There are SSI-approved work programs that allow people to earn money and still keep their Medicaid benefits.

Individuals are eligible for Social Security Disability Insurance (SSDI) if they have contributed to the system and have accrued at least six quarters of credit from employment. They cannot be engaged in full-time work; benefits are based on how much one paid in. Medicaid coverage may begin 29 months after eligibility for cash benefits, but there are no payments for the first five months one is disabled.

To be eligible for SSI and SSDI, individuals must meet low-income and low asset/low resources requirements. If the name of a potential SSI recipient is on a bank account, this money is considered a resource. The only way for money to be exempt is to be in a Special Needs Trust, but Sufian recommended against this if the child will not receive SSI or SSDI benefits, because it severely limits the use of money.

The Affordable Care Act, which ends pre-existing condition exclusions, offers premium assistance to many, removes caps on coverage, and extends children’s ability to remain on parents’ policies is, “the best law ever for people with CF,” said Sufian.

Education laws that protect those with CF include the Individuals with Disabilities Act and Section 504 of the Rehabilitation Act, which protect students in public school, including colleges and universities, and mandate modifications and accommodations such as extensions on homework and exams, private dorm rooms, reduction in workload, and the provision of in-home instruction. It is important to create a plan with the school prior to getting sick. Another resource is the Vocational Rehabilitation Act, which provides federal funding for those with CF to go to college.

Sufian strongly encouraged those needing help navigating the system to contact her team. “We have won every case of Social Security appeals that we have taken on, and we offer free representation to those with CF in Social Security and continuing disability cases.” To contact her, call 1.800.622.0385 or email cflegal@sufianpassamano.com.

At the Crossroad of Advancing CF Lung Disease: Treatment Options
By Linda Burks, EdD

As we celebrate historic advancements in cystic fibrosis (CF) therapies, the mortality brought on by the unyielding claws of this ruthless disease must still be addressed. To prepare CF families facing this eventuality, Elika Rad, MS, RN, NP-C, from the Adult Cystic Fibrosis Center at Stanford Medical Center, clearly described treatment options for those with advanced disease at CFRI’s National CF Family Education Conference.

Mortality in cystic fibrosis is primarily caused by worsening lung disease, followed by respiratory failure. While advising against placing too much emphasis on “numbers,” Rad reminded us that CF survival strongly correlates with FEV1 scores. She stated that people with CF must consistently practice adherence to their medical regimen. However, while this may increase longevity, there is no cure for cystic fibrosis, and...
transplantation may become the only option for survival.

Advanced CF is the third most common reason for lung transplantation. Transplant referral is usually made when FEV1 scores are less than 30% of the predicted value, or in cases of rapid progressive respiratory deterioration. This is especially true for females with repeated episodes of exacerbation, hemoptysis and pneumothorax.

The decision to be listed for transplant is a delicate one: the patient must be sick enough to need new lungs but well enough to survive the surgery. Rad cautioned against being listed too early because transplant is not a cure, but rather, “resetting the clock.” Some clear indicators for transplant referral include oxygen dependency, chronic carbon dioxide retention, worsening pulmonary hypertension, and mechanical ventilation.

Medical barriers to transplant include age, heart disease, major organ failure, cancer, and chronic infection (hepatitis, HIV and high-risk microorganisms). Low socioeconomic status is often associated with worse health outcomes and increased mortality. The huge treatment burden post transplant necessitates excellent adherence, and two primary contraindications for lung transplant are inadequate social support and noncompliance with medical regimens.

Ultimately the patient makes the final decision to accept or decline being listed for transplant. Possible reasons for a patient to decline transplant include personal/religious beliefs, depression or anxiety, and health literacy. The medical team must respect belief systems, while exploring the patient’s reasons for declining transplant. Those with advanced CF lung disease are far more likely to experience depression and anxiety, leading to poor adherence, lower pulmonary function, increased hospitalizations, and decreased quality of life. The team must assess whether the patient’s depression is causing them to say “no.” Finally, the team must explore whether a “no” decision reflects a lack of medical awareness or confidence.

Should advancing lung disease cause severe pain, Rad recommended palliative care, which signifies relieving or lessening symptoms, and involves a strengthened support system, faith or purpose in life, psychological care to help the patient cope, and physical care to reduce painful symptoms. When it becomes clear that there is no chance of survival, hospice care is available once treatment has ceased.

Providers, caregivers and patients can create barriers that delay palliative care; initiating the discussion may be uncomfortable for providers and too stressful for caregivers. Patients often hope they will recover, and may cope through avoidance. Before reaching this point, patients should think ahead and prepare an advanced healthcare directive. When it becomes time to address end-of-life issues, Rad gently stressed that CF adults must be allowed to make their own treatment decisions.

Living with cystic fibrosis takes courage; facing advanced disease takes even more. Just as clinicians give detailed directions in caring for a newly diagnosed child, Elika Rad shared steps to care for those with advanced CF. Until a cure is found, it is comforting to know that there are supportive protocols in place.
CFRI’s 28th National CF Family Education Conference
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Reversing the Vortex: The Inspirational Chris Kvam

By Darlene Batchelder

Chris Kvam, JD, MPP, was diagnosed with cystic fibrosis (CF) when he was four years old, but he does not define himself by his diagnosis. Now 35, he is an assistant district attorney in Rochester, New York, a spouse and an athlete. The opening speaker at CFRI’s 28th annual National CF Family Education Conference, Kvam presented, “Reversing the Vortex: Coping Skills for People with Cystic Fibrosis and their Families.” An energetic and inspirational speaker, Kvam emphasized the importance of exercise, personal challenge and goal setting as a way to motivate adherence and maintain one’s physical and psychological wellbeing.

Kvam’s personal story serves as an excellent example of effectively coping with CF. After his diagnosis, Kvam was never treated differently than his siblings – he was expected to perform well at school, participate in sports, and follow through with household activities as if he did not have CF. As a result of his refusal to let CF define him, he has met many life challenges and experienced many rewards all while maintaining his health: completing a marathon, an Iron Man Triathlon and many cross-country ski marathons, as well as hiking and cycling thousands of miles all while earning his Master’s and law degree, pursuing a demanding career, and enjoying a happy marriage.

He offered the following five tips for parents and people with CF:

• Maintain a positive self-image; you have CF but it does not have you! Sickness is not the default.
• Set healthy short, medium and long-term goals. Even if you don’t achieve them, simply working toward them is a victory. And when you do achieve them, nothing can take away your accomplishments. Remember that the journey is more important than the finish line.

Fully Alive: Physical Activity, Exercise & Yoga for CF

By Siri Vaeth Dunn MSW

“What does being fully alive mean to you?” asked Scott Russell, PT, DPT, of attendees at CFRI’s 28th annual National CF Family Education Conference. Dr. Russell, clinical faculty member at the University of Southern California Division of Bio-kinesiology and Physical Therapy, and lead physical therapist at Keck Medical Center at the University of Southern California, outlined elements of healthy living, including an active lifestyle, exercise, mindfulness, a nutritious diet, social bonding, faith, and adherence to one’s medical regimen, while emphasizing the undeniable benefits of exercise for those with cystic fibrosis (CF).

The current recommendation for an active lifestyle is 10,000 daily steps – the rough equivalent of five miles. Dr. Russell stressed that, “any increase in activity is hugely beneficial.” No matter how little one exercises now, it can be built upon.

Aerobic exercise impacts the heart, lungs, capillaries, and muscle mass. In CF there are often limitations due to the “pulmonary mechanics” of the diaphragm and respiratory muscles. While those with CF often have the sensation that they cannot inhale, the primary issue is exhaling, due to airflow obstruction and air trapping. A negative cycle ensues in which one develops dyspnea, or breathlessness, leading to avoidance of physical activity, leading to muscle weakness that increases breathlessness. Dr. Russell demonstrated “pursed lip breathing,” a technique that can help people to control their respiration while exercising.

Dr. Russell addressed the issue of CF related diabetes (CFRD), noting that average lung function among adults with CFRD is 52% of predicted, as opposed to 72% for those without CFRD. Physical activity is especially important for those with CFRD, as moderate intensity exercise creates a positive immune response, often decreasing inflammation and the risk of respiratory tract infections. High intensity training is the primary way to increase fitness. Research conducted with people facing end-stage lung disease indicates that it is safe when done in a supervised setting and may decrease dynamic hyperinflation and air trapping. While pulmonary function plays a role in predicting physical fitness, adaptations to heart and vasculature...
The Inspirational Chris Kvam  
Continued from page 11

- Create a sustainable lifestyle that includes exercise. Push yourself to discover your limits by competing with yourself rather than some ideal. Experiment with new activities and find ones you enjoy. Find ways to stay committed.
- Create, welcome and nurture support from family, friends and significant others.

In doing so, you allow them to give you a gift you need when you can’t do everything yourself.
- Having cystic fibrosis is a demanding, time consuming, and often painful journey, so nurture empathy for yourself and others, as well as compassion and love. These ways of being will enrich the journey.

Kvam cares deeply about finding ways to help people with CF cope with the many challenges of the disease. Said Kvam, “Adherence, quality of life and mental health issues associated with CF interest me as much as advances in current science.” Kvam has participated actively in the development of mental health assessment tools and support for those with CF. “Living fully with CF requires the development of coping skills and an appreciation of the whole person with CF, not as a patient defined by a condition.”

Fully Alive: Physical Activity  
Continued from page 11

play a role. Those with CF often have lower muscle mass, likely due to chronic inflammation, ventilatory issues and decreased activity. There are many tests to assess bone and joint health and flexibility. Despite improvements in CF care, those with CF have lower bone mineral density. Strategies to improve bone health include weight lifting and impact exercise. Balance training can be key for those with CF, as posture becomes malaligned, often due to ototoxicity from antibiotic treatments.

Dr. Russell reviewed the positive results from his multi-center study investigating whether yoga impacts the postural system of those with CF. Yoga positions were modified to minimize the risk of gastroesophageal reflux (GERD), which can damage the lungs. Participants showed significant improvement in chest wall excursion and scapular position (how far forward one holds the shoulders) as well as improved leg strength. While there was no change in FEV1, participants’ balance and body image improved. Because yoga is a low-to-moderate intensity exercise, it is an excellent place to begin one’s exercise intervention. Based on the results, Dr. Russell hopes to conduct a future randomized control trial with a larger sample size.

In conclusion, Dr. Russell stressed, “Sit less, and do more. Make exercise something that you want to do, and try to do a little more each day. Be fully alive.”

Recognizing Our Extraordinary Community Heroes  
By Siri Vaeth Dunn, MSW

Each year at our National Cystic Fibrosis Family Education Conference, CFRI honors individuals that have made extraordinary contributions to the cystic fibrosis (CF) community.

The 2015 Dave Stuckert Memorial Volunteer of the Year Award, given to a community member who actively volunteers for CFRI, was presented to Doug Modlin, PhD. Doug has served on CFRI’s Research Advisory Committee for over 30 years, since his daughter’s diagnosis with CF. He has served on CFRI’s Board of Directors since 2014 and is the chair of the Strategic Planning Committee and the Research Strategic Planning Committee. Doug is tireless in his support of CFRI, his quest to support the search for a cure, and his advocacy for all those impacted by the disease.

The 2015 Professional of the Year Award was presented to Deborah “DJ” Kaley, RN, MSN, Pulmonary Nurse Case Manager at Bay Area Pediatric Pulmonary group, who has dedicated herself to meeting the needs of her CF patients and the CF community for nearly 40 years. In addition to her excellent patient-oriented clinical care, DJ serves on CFRI’s CF Quality of Life Committee and Medical Advisory Board, and also serves as a mentor to Nurse Coordinators at other CF centers.

Anna Modlin, MA, received the 2015 CFRI Partners in Living Award in Memory of Anabel Stenzel. Anna is 34 years old with CF and had a lung transplant in 2010. Anna has volunteered on the CFRI Retreat Committee for 16 years, and is a regular conference participant. She is a board member for Richie’s Spirit Foundation, which promotes organ donation and CF awareness. Anna competes in the Transplant Games of America and the World Transplant Games, and exemplifies Anabel’s spirit through her courage, positive attitude, and volunteer service to CFRI.

Congratulations to these outstanding individuals!

Anna Modlin, MA  Doug Modlin, PhD  Deborah “DJ” Kaley, RN, MSN
Keys to a Successful Transition  By Amy Baugh

“Cystic fibrosis (CF) is not a pediatric disease anymore,” stated Dr. Gregory Sawicki, Associate Director of the Cystic Fibrosis Center at Boston Children’s Hospital and Assistant Professor of Pediatrics at Harvard Medical School. A national expert in the area of CF transition, Dr. Sawicki helped to develop a transition readiness survey tool for youth with chronic diseases that is currently used in health care transition studies throughout the country. In his enlightening presentation at CFRI’s 28th National Cystic Fibrosis Family Education Conference, Dr. Sawicki stressed that as the median life expectancy of CF patients continues to increase, the transition between pediatric and adult care must be thoughtfully addressed.

Dr. Sawicki discussed the challenges posed by the transition period between adolescent and adult care. Adolescence remains a high-risk period; during this time disease progression often significantly accelerates. Teenagers with CF face the same issues as their peers – no longer children but not yet responsible adults. Having mostly relied upon their parents, some teenagers avoid personal responsibility for their CF care. They often minimize symptoms, have busier schedules and are less adherent with treatments. Furthermore, they may harbor anger towards the disease and retaliate by not following their regimen. Dr. Sawicki noted that a key factor to helping teens through transition is recognizing that there is no universal teenage behavior and each patient responds uniquely.

“Transition does not equal transfer,” stressed Dr. Sawicki. Historically, CF patients were given a packet of information that described adult care and then transferred out of pediatrics, even when they had not emotionally, mentally, financially, or socially transitioned to being responsible for their care. Transfer is not the beginning or the end; it is merely one small part of a much larger process that includes many steps; preparation, discharge and handoff are just a few of them. While many CF programs may be very good at some of these steps, rarely are programs good at all of them.

People often associate a decline in health with transfer to adult care, but when done properly, the data proves otherwise. Findings from the

Singer/Songwriter Tess Dunn Mesmerizes Sunday Conference Guests

By Bridget Barnes

The heart of this year’s 28th National CF Family Education Conference was Tess Dunn’s presentation, “Rose Tattoos & Rock Bands: Living an Engaged Life with Cystic Fibrosis,” which included stories, photos, film, and music. Introduced by CFRI’s Executive Director Sue Landgraf, Dunn was described as a “joyful young woman,” and thanked for “bringing meaning and purpose to others living with cystic fibrosis (CF).”

“I am here to talk honestly about my experience living with CF and share what has helped me through the challenges,” said Dunn, who outlined five key ingredients to an engaged life: 1) Find your passion; 2) Surround yourself with people who support you and your passion; 3) Celebrate choices large and small; 4) Allow yourself to go dark from time to time; 5) Create a Plan B that’s as good as your Plan A.

Dunn was born in 1994 and, “I seemed like a perfectly healthy baby in the hospital, but when we came home I was hungry all the time and cried a lot.” After five months, doctors determined Dunn had CF; she had developed pneumonia and weighed barely 10 pounds. About her medical regimen, Dunn noted, “This was my ‘normal’ and I never knew anything different.”

Having cystic fibrosis remained ‘normal’ for Dunn until adolescence, and then “my world changed.” Middle school was full of social challenges and classmates who made Dunn feel different and isolated.

It was at this point that music saved her. Dunn had found her passion, which she described as her “private outlet and public therapy.” The Make-A-Wish Foundation supported the recording of the first of her three albums, “Darling Just Walk,” which was life-changing as she heard her own songs expand in the studio and felt tremendous support and camaraderie from family, bandmates and friends.
Keys to a Successful Transition  
Continued from page 13

CF Foundation registry show that there is no difference in body-mass index (BMI), lung function, or number of hospitalizations during the transitional period. These findings should help mitigate fears of parents and CF patients as they approach transition.

Dr. Sawicki interviewed adolescents with CF and their parents at three care centers, and discussed living with CF, daily self-management routines, and views on transition and transfer. In general, both the adolescents and their parents expressed concern over the transition process and the accompanying developmental challenges. Sawicki found reoccurring themes surrounding the need for transition program support, and written care plans for ease of access to medical records. Because transition is critical and complex, the process should start during the pre-teen years to provide greater opportunities to address and resolve concerns around transfer before the CF patient is a young adult.

Recognition of the importance of successful transition has led to the creation of tools to aid with the process. For example, www.gottransition.org provides steps through six core elements of transition, and has numerous checklists that are beneficial for those going through the process. Another resource, CF R.I.S.E., offers a tool kit that includes online assessments of both skills and knowledge that is being used by several CF centers. In conclusion, Dr. Sawicki strongly emphasized that, “transition is a continuum, not a singular point in time.”

Singer/Songwriter Tess Dunn  
Continued from page 13

With the opportunities and exposure created by her music, Dunn became a vocal CF advocate who now performs extensively – including benefit concerts for CFRI – and spreads CF awareness.

In “Celebrating choices, large and small,” Dunn acknowledged that her mother “always validated the burdens that CF places on me and helped find other areas where I could have control…such as hair color!” When her mother said to critics, “You can’t change CF, but you can change your hair,” Dunn knew that “she was my ally.”

Shedding light on “allowing yourself to go dark from time to time,” Dunn said, “Sometimes being an advocate and source of inspiration is hard. There are many days when I feel the opposite. You have to give yourself time to rest and just be.” A rose tattoo on Dunn’s leg honors the memory of Gianna Rose Altano, who passed away at age 23 waiting for a lung transplant. “My tattoo says to the world: hear Gianna’s story and help support the search for a cure.”

Dunn’s final point, “Create a Plan B that’s as good as your Plan A,” is a reality for her. While she would love to sign with a major record label, she is also earning a degree in audio engineering to become one of few female sound engineers in the music industry. “I love my Plan B as it means I don’t have to give up on my Plan A.”

With a wide smile and a wide-open heart, Dunn closed her presentation with Matt Scales’ haunting song “Breathe,” and then flowed effortlessly into a sing-a-long of Leonard Cohen’s “Hallelujah.” There was not a dry eye when the inspiring Dunn exited the stage to a standing ovation.

CFRI CF Summer Retreat:  
A Week of Priceless Connection  
By Todd Giebenhain

This year at CF Summer Retreat we tried something we’d never done before. Throwing caution to the wind, we deliberately bumped into each other: many times, and with giddy abandon. It was both delightful and a long time coming. Maybe we looked ridiculous running around in goofy inflatable plastic donuts with our legs sticking out. But this allowed us, for the first time, to touch each other in a sense - and then literally and immediately go head over heels in somersaults as a consequence.

Many thanks to our friends at Planet Xone for their generous donation of the bubble balls

This year’s CF Summer Retreat offered many things: Fascinating educational presentations, physical activities, art, and supportive “rap” groups. But the bubbles were a special treat. People who have personal affection for each other, yet who are advised against conveying it physically with even a hug or a handshake because of potential cross-infection risks, had a brief window of time to safely smash each other and laugh and cough their brains out. This awareness was a profound side effect that settled in after the fact, beyond the fun bit of blood-pumping exercise. What can I say, like the rest of the retreat, it hit the spot. Red rover red rover, send another week of priceless connection right over.
Cruise for a Cure: When CFRI community members book their passage on the May 11, 2016 11-day Crystal Cruise from Istanbul to Rome, for every booking made, All Cruise Travel will generously donate $550 back to CFRI.

Charitable Planned Giving: Planned giving offers benefits for donors that often include increased income and substantial tax savings, while providing the opportunity to meet your philanthropic goals and provide positive tax benefits.

Attend a CFRI Fundraising Event: No matter what type of event you enjoy, we have one for you! We regularly have concerts, golf tournaments and other events. Have fun while supporting CFRI.

Mothers’ Day Tea - Ongoing: Our Mothers’ Day Tea is CFRI’s largest fundraiser, and operates year round! Please contact us if you would like to become a Tea Sender – you can send invitations, or participate online. It is fast, easy and fun!

Coffee for a Cure: Through Coffee for a Cure, you invite your family members, friends and colleagues to join you for a virtual cup of coffee, while supporting CFRI and its vital programs for the CF community.

Giving Gifts of Stock to CFRI: When giving a gift of appreciated stock to CFRI you will not pay capital gains tax, and you will receive an income tax charitable deduction for the fair market value of the stock on the date of the gift. If you wish to donate stock certificates to CFRI, please contact us for instructions.

Hold Your Own Fundraiser: Big or small, we appreciate them all! Zumbathons, concerts, cupcake sales, dinner parties with a special guest of honor, bocce ball tournaments – come up with an idea and we will support you!

Tributes in Honor of and in Memory of: Any gift to CFRI can be made in honor or in memory of a loved one. Your loved one’s name will appear in our newsletter, CFRI Community, and if requested, an acknowledgement will be sent to the person you designate.

Vehicle Donations: If you have a car, boat, RV, or motorcycle that you no longer need, please consider donating it to CFRI. This contribution is tax-deductible; we will coordinate the transfer. Visit our web site for details.

For more information please contact Mary Convento at 650.404.9975 or mconvento@cfri.org

Becoming a CFRI Partner in Living! Your Support Can Change Lives

31st Annual Golf Tournament Benefitting CFRI A Phenomenal Success!

On Monday, August 3, at the illustrious Pasatiempo Golf Club – a “top-100” course – 123 golfers gathered to help find a cure for those with cystic fibrosis (CF). Before the day was done, their numbers had grown to 150, who together raised over $55,000 to support CFRI’s research, education and support programs. The event is personal for Scott Hoyt, manager of the legendary Pasatiempo Golf Club, who has two daughters with CF, and who dedicated many years serving as treasurer on CFRI’s Board of Directors. Said Scott, “What makes this tournament so special is the exact same thing that makes CFRI special and unique. It is a personal, caring and supportive event. Each year, 85% of the participants return because they have a personal connection to either an individual with CF or a caregiver.”

Once again, one of the largest groups in attendance, “Team Becca,” was there in support of Becca Roanhaus, daughter of long-time CFRI Board member Mike Roanhaus. The group included many of Mike’s fraternity brothers from college. Major sponsors of the event included the Mike and Dea Roanhaus family, HDR Architecture, Star One Credit Union, the Kirkorian Family Foundation, and the Scott and Anne Hoyt family.

CFRI is extremely grateful to Scott Hoyt, the dedicated members of the golf committee, and the many participants who are helping support cutting-edge research and much needed support programs for those living with CF.
The need for increased research funding is critical. As we stand on the cusp of new therapeutic breakthroughs in cystic fibrosis (CF), CFRI needs your help to continue this forward momentum and move us closer to a cure. Please give a gift to CFRI; it will be matched 1-to-1 by our Jessica Fredrick Memorial CF Research Challenge Circle, thereby doubling the impact of your donation!

There have been extraordinary advances in CF treatment options, but there is still no cure. Our CF Research Challenge Fund is named in memory of Jessica Fredrick, an inspiring young woman who passed away last year at the age of 21 from complications of cystic fibrosis. After her death, Jessica’s aunt, Suzanne Freiley, gave CFRI the largest individual donation in our history, with the goal of inspiring others to give generously to fund CF research.

Jessica reminds us of the urgency facing those with CF, who spend hours each day coping with lung disease, liver issues, diabetes, pancreatic insufficiency, and other complications. We have more work to do to improve treatments and find a cure, and we cannot do it without you. Unless otherwise requested by the donor, all gifts received through this newsletter will be matched 1-to-1 and used to fund vital CF research projects.

Please join with us to improve the lives of those with cystic fibrosis.

To contribute to the Jessica Fredrick Memorial CF Research Challenge fund, please use the enclosed envelope, call 650.404.9975, or email cfri@cfri.org

For their generous support of CFRI Community, special thanks to:
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