

CFRI *news*

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The Importance of Anti-Inflammatory Therapies in the CF Treatment Regimen

By Siri Vaeth-Dunn

A baby girl is born with cystic fibrosis, and at that moment her lungs and airways appear to be clinically normal. Almost immediately, however, the secretions in her lungs begin to cause mucus plugging and inflammation. In response, her body sends neutrophils and proinflammatory mediators to cope. The dying cells litter her airways with their DNA and necrotic material, while releasing high amounts of interleukin-8 (IL-8), and elastase, both of which have been found to damage airway tissue. Thus begins an ongoing and debilitating cycle of airway inflammation.

Because airway inflammation is a key element of cystic fibrosis (CF) lung disease, researchers and clinicians are focusing their efforts on identifying and administering

anti-inflammatory therapies that are both safe and effective. There is a direct correlation between high neutrophil counts – a type of white blood cell that is among the first to respond to infection or inflammation - and decreased lung function. Studies utilizing bronchoalveolar lavage (BAL) with CF infants have found very high concentrations of neutrophils in the airways, even when there are no identifiable pathogens. When bacteria are present, the inflammatory response is even greater. BAL samples from CF lungs consistently find neutrophil counts that are 20 to 100 times higher than those found in healthy lungs.

In CF lungs, abnormal CFTR function leads to diminished water content of the airway surface liquid layer, causing the thick mucus that provides a perfect environment for opportunistic bacteria

and inflammation. Anti-inflammatory therapies have been utilized for many years, but their use is increasing as numerous research studies further illuminate the dangers of chronic inflammation in the lungs, and the benefits of actively addressing this issue.

Well-known anti-inflammatory drugs include corticosteroids, ibuprofen and azithromycin, all of which have been studied in clinical trials. While oral steroids (most commonly prednisone) were found in early clinical trials to increase weight gain and improve lung function, the adverse effects of long-term use outweigh the benefits. These include the inability to maintain and put on weight, osteoporosis, high blood sugars, cataracts and a decreased ability to fight infections. Inhaled corticosteroids are widely prescribed, but

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RESEARCH

Induced Pluripotent Stem Cells: A Recent Breakthrough In Regenerative Medicine

By Dieter C. Gruenert, Ph.D.

The development of a comprehensive therapy for cystic fibrosis (CF) has been elusive, since there are multiple elements to providing an efficacious therapy. Not only is it necessary to correct the ion transport defect in CF through pharmacological and/or genetic interventions, it is also necessary to repair the tissue and organs that have been damaged as a result of the progression of the

disease. Recent studies in regenerative medicine have suggested that this might be possible through the use of stem cells.

Stem cells have the unique property of self-renewal as well as the ability to convert (differentiate) into different cell types within the body. Stem cells that are derived from various organs/tissue are classified as adult stem cells (ASCs) and these are generally recruited to replace tissue that has been damaged or destroyed. In



Dieter Gruenert, Ph.D.

addition to their ability to regenerate tissue within the organ where they reside, ASCs have been shown to be multi-potent and have the ability to become many, but not all

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2672 Bayshore Parkway, Suite 520
Mountain View, CA 94043
Phone: 650.404.9975
Fax: 650.404.9981
Email: cfri@cfri.org
Website: www.CFRI.org

Editorial Chief
Carroll Jenkins

**Contributing Editors/
Newsletter Committee**
Siri Vaeth-Dunn
Bridget Barnes
David Soohoo
Mary Convento

Contributing Writers
Siri Vaeth-Dunn
Bridget Barnes
Carroll Jenkins
Dieter Gruenert, Ph.D.
Ann Robinson
Hal Soloff, J.D.
Laura Tillman

Graphic Designer
Kathy Mitchell

Photographer
Craig Burleigh

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Website Development
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Notes from our Executive Director

I am pleased to report that CFRI has earned top reviews on both Charity Navigator and Guidestar this year. We are recognized for serving the CF community with high integrity and for being incredibly responsive as we connect CF resources to community needs.

In this issue of CFRInews, we present a rich menu of topics. Researcher Dieter Gruenert offers insight into new stem cell developments. Bridget Barnes shares the latest updates on ataluren (PTC124). And Siri Vaeth-Dunn gives an informative overview of the importance of anti-inflammatory therapies, starting at an early age.

Laura Tillman cites several useful articles in her discussion of CF and Sleep. Siri brings us a very thoughtful article on Teens and Cystic Fibrosis: Their Quest for Healthy Independence, and also reviews our spring CF Discovery Series™ presentation on CF and Emotional Health.

Former CFRI Executive Director, Ann Robinson, gives us new information on the CF Quality of Life Program: A Living Legacy of Peter Judge. This is a wonderful service for those who would like support as they face the many challenges that cystic fibrosis presents.



Meet Eric Marten, a talented, energetic volunteer who has brought our organization into the webcast era with CFRIlive! You are likely to see him at our summer Conference and Retreat – check this issue for more information on both (and capture the early-bird specials!). Thank you to our sponsors for helping us provide these two outstanding events.

In closing, I would like to tip my hat in recognition of Hal Soloff, who turned 80 this spring, despite having cystic fibrosis. CF has been a challenge, but has not held him back from eight decades of accomplishments and adventures. He continues to be a mentor and advocate for all who live with cystic fibrosis.

I hope to see you in July!

Warmly,

Carroll Jenkins
Executive Director
650.404.9977

CFRI and CFI

By Carroll Jenkins

This past February, I was able to participate in a highly engaging and informative Annual Winter Education Retreat, hosted by the Cystic Fibrosis Institute near Chicago. Their organization, like ours, supports those affected by cystic fibrosis.

This year's event was centered around the theme "A Medical Mystery Tour," and offered workshops on CFRD, airway clearance, exercise, CF genetics and more. Experts Marlyn Woo, M.D., and Samya Nasr, M.D. were among the speakers who made presentations, and I was invited to speak as well.

CFI presents their Hans Wessel Award for "lifetime commitment to cystic fibrosis" at this annual conference. This year, I was honored



Carroll Jenkins and Steven Boas, M.D.

to accept the award on behalf of CFRI. It is powerful when organizations collaborate in support of those who are challenged by cystic fibrosis.

CEO and founder of CFI, Dr. Steven Boas believes in the importance of education, advocacy and awareness for the CF community. As Medical Director of the Cystic Fibrosis Center of Chicago (<http://www.chicagocfcarecenter.org>), Dr. Boas

actively participates in both independent and national clinical research involving the newest therapies available for children with asthma and cystic fibrosis.

For more information, visit

www.cysticfibrosisinstitute.org.

Will Ataluren Make Sense of Nonsense Mutations in CF?

By Bridget Barnes

A new investigational drug called ataluren may end up making sense of nonsense mutations in cystic fibrosis. Developed by PTC Therapeutics, Inc., ataluren, formerly known as PTC 124, is an oral drug designed to restore the production and enable the formation of a functioning protein in cystic fibrosis (CF) patients who have what is known as a “nonsense” or “stop” mutation. Approximately 10% of people with cystic fibrosis have “nonsense” mutations, which interrupt the production of the cystic fibrosis transmembrane conductance regulator (CFTR), thereby halting the synthesis of this essential protein. Recent published data regarding a Phase 2a clinical trial of ataluren in children and teens with nonsense mutation cystic fibrosis (nmCF) show that treatment with ataluren improved lung function, reduced coughing and resulted in statistically significant improvements in the production and function of CFTR. “We are encouraged by the results in this study, which show that ataluren is pharmacologically active and generally well tolerated in children with nonsense mutation cystic fibrosis,” said Isabelle Sermet-Gaudelus, M.D., Ph.D., and Principal Investigator at l’Hôpital Necker-Enfants Malades in France. “These safety and activity data in pediatric patients support the inclusion of children in long-term studies of ataluren.”

People with CF lack adequate levels of the CFTR protein, a chloride channel necessary for the normal function of the lung, pancreas, liver and other organs. In nmCF, an interruption in the genetic code known as a nonsense mutation prematurely stops the synthesis of CFTR, causing the protein to be short and non-functioning. Ataluren is designed to overcome the nonsense mutation and enable the production of a full-length functional CFTR protein. A simple genetic test can determine if a person’s disease is caused by a nonsense mutation.

The randomized Phase 2a dose-ranging study was designed to evaluate the safety and efficacy of two ataluren doses in children with nmCF. Based in Belgium and France, the study enrolled 30 partici-

pants 6 to 18 years of age, with a nonsense mutation in at least one allele of the CFTR and abnormal baseline epithelial chloride transport. The primary goal of the Phase 2a trial was to measure CFTR chloride transport by assessing nasal transepithelial potential difference (TEPD) in the nose for the presence and activity of the CFTR protein. Epithelial cells in the nasal passages perform the same function as they do in the lungs. Results showed that ataluren induced statistically significant improvements in chloride channel activity, with some patients achieving chloride transport values in the range of healthy children. Overall, 50% of the participants had a total chloride transport response and importantly, TEPD compliance was excellent, suggesting repeated TEPD evaluations are feasible in children of this age group.



Across all patients, there was a 17% improvement in the proportion of nasal epithelial cells showing CFTR protein expression. In addition, efficacy results showed that multiple nonsense mutation CF genotypes responded to ataluren therapy. Safety results showed that ataluren was generally well tolerated and compliance was greater than 93%. Adverse events were mild or moderate, and no patients discontinued treatment due to an ataluren-related adverse event.

Based on the positive results from the Phase 2a study of ataluren, PTC Therapeutics, Inc. recently completed enrollment of a Phase 3 clinical trial, which is expected to conclude at the end of this year. The 48-week trial is designed to determine whether ataluren can improve the lung function of patients with nmCF and reduce the symptoms associated with CF, and is intended to provide the key information necessary for the U.S. Food and Drug Administration (FDA) to evaluate its safety and efficacy. The trial has enrolled 238 patients at 36 sites in the United States, Europe and Israel. “We are pleased to

have completed enrollment of our second pivotal clinical trial of ataluren in patients with a nonsense mutation genetic disorder. This is a tremendous achievement and testament to the commitment of clinical trial patients and their families, as well as investigators and trial site staff,” said Stuart Peltz, Ph.D., and President and CEO of PTC Therapeutics, Inc.

“The enrollment of this trial represents an important step forward in our efforts to develop disease-modifying treatments that advance the standard of care in CF and improve quality of life for CF patients,” stated Michael Konstan, M.D. and Chairman of the Department of Pediatrics at Rainbow Babies and Children’s Hospital in Cleveland, Ohio. Dr. Eitan Kerem, Director of the Pediatrics and CF Center at Hadassah University Hospital in Jerusalem, Israel, added, “Despite significant advances in the 21 years since the identification of the disease-causing gene, cystic fibrosis remains a debilitating and life-threatening disorder, and available therapies focus only on alleviating symptoms. ataluren couples a patient’s genetic diagnosis with a mutation-specific therapeutic approach designed to address the underlying cause of the disease.”

The FDA and the European Commission have granted ataluren “Orphan Drug” status (a U.S. designation for drugs developed for diseases affecting fewer than 200,000 people) for the treatment of nonsense mutation cystic fibrosis, and nonsense mutation Duchenne and Becker muscular dystrophy. The FDA has also granted ataluren “Subpart E” designation for expedited development, evaluation and marketing for CF. The development of ataluren has been supported by grants from the Cystic Fibrosis Foundation Therapeutics Inc., FDA’s Office of Orphan Products Development, Muscular Dystrophy Association, National Center for Research Resources, National Heart, Lung, and Blood Institute, and Parent Project Muscular Dystrophy.

For further information on ataluren clinical trials visit: www.clinicaltrials.gov/NCT00458341 and www.cff.org.

CF and Sleep

By Laura Tillman

Sleep is defined as a condition of the body and mind in which the nervous system is relatively inactive, the eyes are closed, the postural muscles are relaxed, consciousness is reduced and there is a decreased ability to react to stimuli. It is a naturally recurring state in which there is growth and rejuvenation of the immune, nervous, skeletal and muscular systems.

However, patients with cystic fibrosis (CF) report poor sleep quality. Although cough plays an important role in the sleep disruption of CF patients, it is unlikely that it is the sole contributor to sleep disturbance. Other factors which may contribute to sleep disturbances in cystic fibrosis include nocturnal wheezing, as well as side effects

of medications and chronic pain. Increased work of breathing associated with severe airflow limitation may also contribute to sleep disruption as increasing ventilatory effort is known to cause arousal from sleep.

There appears to be a relationship between the severity of lung disease and sleep disturbance, as significant changes in ventilation and gas exchange during sleep occur in those with severe lung disease. Pulmonary exacerbations are common and associated with poorer sleep. It is likely that sleep disruption contributes significantly to fatigue, excessive daytime sleepiness and cognitive impairment by causing a low concentration of oxygen in the blood.



Noninvasive ventilation has been shown to improve symptoms, reverse slow rate of breathing and increase exercise capacity in CF patients.

Articles That Further Discuss The Above Include:

Cystic Fibrosis Patients Have Poor Sleep Quality Despite Normal Sleep Latency and Efficiency.

Jankelowitz, Larry; Kathryn J. Reid; Lisa Wolfe; Joanne Cullina; Phyllis C. Zee; Manu Jain.
CHEST. May 2005. 127(5): 1593-99.
<http://tinyurl.com/3syo4nn>

Night-to-Night Variability in Sleep in Cystic Fibrosis.

Milross, M.A.; A.J. Piper; M. Norman; G.N. Willson; R.R. Grunstein; C.E. Sullivan; P.T. Bye.
Sleep Med. May 2002. 3(3): 213-19.
<http://tinyurl.com/cy2abu>

Nocturnal Hypoxia and Sleep Disturbances in Cystic Fibrosis.

de Castro-Silva, C.; V.M. de Bruin; A.G. Cavalcante; L.R. Bittencourt; P.F. de Bruin.
Pediatr Pulmonol. November 2009. 44(11): 1143-50.
<http://tinyurl.com/6k7t9pa>

Sleep Disturbances in Cystic Fibrosis.

Mattewal, Amarbir S.; Shyamsunder Subramanian.
Current Respiratory Medicine Reviews. November 2009. 5(4): 230-33.
<http://tinyurl.com/yhe6s5q>

Sleep Quality and Daytime Function in Adults with Cystic Fibrosis and Severe Lung Disease.

Dancey, D.R.; E.D. Tullis; R. Heslegrave; K. Thornley; P.J. Hanly.
European Respiratory Journal. March 2002. 19(3): 504-10.
<http://tinyurl.com/3poe5y2>

Subjective Sleep Quality in Cystic Fibrosis.

Milross, M.A.; A.J. Piper; M. Norman; C.J. Dobbins; R.R. Grunstein; C.E. Sullivan; P.T. Bye.
Sleep Med. May 2002. 3(3): 205-12.
<http://tinyurl.com/45xz924>

The Relationship Between Sleep Disturbance and Pulmonary Function in Stable Pediatric Cystic Fibrosis Patients.

Amin, Raouf; Judy Bean; Kathleen Burklow; Jennifer Jeffries.
CHEST. September 2005. 128(3): 1357-63.
<http://tinyurl.com/3d5svvr>

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"Cystic Fibrosis in the Spotlight: Taking a Leading Role"
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Redwood City, CA

July 31 – August 7, 2011

CFRI Teen and Adult Retreat
"CFRI High: Learning to Live!"
Vallombrosa Center
Menlo Park, CA

August 1, 2011

27th Annual
CFRI Golf Tournament
Cinnabar Hills Golf Club
San Jose, CA

For more information, contact
CFRI at 650.404.9975 or
CFRI@cfri.org.

A View From The Hill: Hal Soloff Turns 80

By Hal Soloff

There's a saying, "Some people have a life's experience, while others experience life." I can say with a smile on my ancient face, I have experienced life!

When I was born on April 9, 1931, we lived with my grandparents here in Connecticut and the Great Depression had begun. I've always said, the great depression is what my mother had when she first saw me!

As a newborn, I was discharged from the hospital even though I was jaundiced, bloated, and continually crying. My mother noted my distress, picked up the telephone receiver, and told the operator the situation (no machine telling you to "dial 911" in those days). The operator was given the name of our family doctor and knew where he played cards that night.



Phyllis and Hal Soloff in 1963

The doctor immediately came to our home. He treated me for what was probably meconium ileus, and saved my life.

Growing up, I always got a lot of attention, love, and the best health care available. I have been hospitalized many times. When I was eight, I spent the summer in Dallas in the hospital being fed via IVs, as I could not tolerate the heat. By the time I was a teenager, I had already endured many polypoid tissue removals, and two Caldwell Luc surgeries. I was not diagnosed with

cystic fibrosis (CF) until I was 28 years old.

I have carried my medications and an inhalation machine wherever I've been, and I have been on antibiotics since 1945. I have had two DNA studies (my mutations are W1282X and A455E), and volunteered for exploratory surgery. I have cultured *Pseudomonas aeruginosa*, *Staph aureus*, and aspergillus. Now I am culturing antibiotic-resistant and quickly debilitating *achromobacter xylosoxidans*. No antibiotic has touched it.

It has not been an easy journey, but I managed to complete two years in the Air Force during the Korean War, law school, a bar exam, a doctorate and two master's degrees. I had a loving marriage to an amazing woman for fifty-three years,

After taking a year's sabbatical from the University of London, my wife and I both earned the title, "Associate, University of London." We traveled and hiked for over forty years through much of Europe and the U.S., including Alaska and Hawaii, where our daughter Jennifer and her husband live. Our son, David, lives in New York.

"Forget mortality statistics, as they are often bogus, and everyone with CF is different."— Hal Soloff, 80 with CF



Phyllis and Hal Soloff in 2009

My beloved wife died of metastasized pancreatic cancer in August, 2009, after completing her "bucket list," with a trip to Hawaii, and to London to celebrate her 75th birthday. In Hawaii, our children took us to every expensive restaurant on Oahu, and I have the receipts to prove it. My wife, Phyllis, was the most beautiful and bravest person I have known, and she is the reason I have survived. Losing my wife and my sister (who died of CF at age 20) are my only regrets.

From my perspective, parents of a child with cystic fibrosis or adults with CF should not make the disease the focal point of their lives. It's a part of life, and goals can be set and accomplished. A parent should show love without anxiety, teach the discipline needed to survive CF (yes, we had two teenagers), and help them to look positively toward living their dreams. Forget mortality statistics, as they are often bogus, and everyone with CF is different. Take charge when dealing with doctors. Ask questions and get clear answers, or fire them. Don't be timid! It worked for me, and I hope it works for you.



Editor's note: In 2007, Hal was recognized by *Heroes of Hope™ Living with Cystic Fibrosis* as "a unique individual with cystic fibrosis who strives to live a full, productive life and serves as a role model to others, while managing his own healthcare needs." That certainly is Hal. Nominate someone you know to be a Hero of Hope (at any age!) today. For more information and to complete a nomination form, go to www.heroesofhope.com.



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Pradeep Singh, M.D., University of Washington, Seattle, WA

"CF Gastrointestinal Problems - What You Can Do"

Ronald J. Sokol, M.D., University of Colorado School of Medicine, Aurora, CO

"Genetic and Environmental Factors in CF Health"

J. Michael Collaco, M.D., John Hopkins Hospital, Baltimore, MD

"An Overview of Sleep Related Problems In Cystic Fibrosis"

Nanci Yuan, M.D., Stanford Hospital, Palo Alto, CA

"Ways to Clear Your Airways"

Maggie McIlwaine, M.C.S.P., British Columbia Children's Hospital, Vancouver, BC

"Cystic Fibrosis Related Diabetes" (CFRD)

Richard Cohen, M.D., Kaiser Permanente, Portland, OR

"Your Health Matters: Being an Effective Advocate"

Tiffany Christensen, Advocate, Coordinator and Author, Raleigh-Durham, NC

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Jordan Herskowitz, CF Sibling, Artistic Director, New York, NY

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Paul Quinton, Ph.D., University of California San Diego, La Jolla, CA

Dieter Gruenert, Ph.D., University of California San Francisco, San Francisco, CA

"Ask the Experts" Panel Discussion

Richard Cohen, M.D., Mike Collaco, M.D., Maggie McIlwaine, M.C.S.P., Pradeep Singh, M.D.
Nanci Yuan, M.D.

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Cystic Fibrosis and Emotional Health

By Siri Vaeth-Dunn

The emotional well being of those living with cystic fibrosis (CF) is critical to their physical health.

The vital importance of addressing the emotional challenges of living with CF was eloquently and sensitively conveyed by Meg Dvorak, L.C.S.W., Adult Social Worker at the CF Center at Stanford, and Justin Birnbaum, M.D., Medical Director of the Behavioral Medicine/Medical Psychiatry Unit at Stanford University Medical Center. Their presentation, “Cystic Fibrosis and Emotional Health” was part of the CF Discovery Series™, co-sponsored by CFRI and Stanford Hospital and Clinics.

Ms. Dvorak outlined the social worker’s role as the only member of the CF team whose sole focus is the whole patient, not just the disease. Social workers conduct ongoing psychosocial assessments in both the clinic and hospital settings and provide interventions tailored to patients’ strengths and needs by providing patient advocacy, counseling services, support groups, assistance with disability applications, and crisis support.

CF presents many emotional challenges for patients and families. There is never a break from the daily treatments. Conversely, those who don’t fully adhere to their medical regimen may feel guilty or face a decline in health. Some may feel stigmatized by their cough, as people who are unaware of their CF think they are contagious. Patients who were diagnosed at a young age may have parents who are overly protective, which when combined with multiple hospitalizations, school absenteeism, and difficulty in being a part of peer activities can delay individuation. Adult developmental milestones may be reached at a later age than siblings and peers.

Studies show that CF patients do not have a higher incidence of psychiatric diagnoses than the general population. However, depression and anxiety are not uncommon among the CF population and, as outlined by Ms. Dvorak, general “themes of depression” include loss, chronic isolation, hopelessness, and grief. Additionally,

depression may lead to poor health care compliance, and is directly correlated to lung function.

It can be challenging for the CF medical team to sort out whether physical symptoms — including fatigue and chronic pain — are a result of the disease or of emotional distress, as these symptoms frequently overlap. Anxiety, triggered by breathlessness, coughing, hospitalization, reduced pulmonary function, the presence of resistant bacteria, finances, insurance issues, worries about the future, and mortality will often manifest in both emotional and physical ways.

Living with CF is stressful. In response, the body releases hormones which can be harmful. Stress also increases exacerbations due to reduced sleep, suppressed appetite, and altered self-care. Ms. Dvorak explored coping strategies for people with CF that can be directly correlated to disease outcomes. Everyone has a unique coping style, which is the result of one’s upbringing, lifestyle, education and personality. Studies show that adult CF patients have a continuum of coping skills. On one end of the spectrum is CF denial, passivity and avoidance; while on the other is CF involvement and optimism. People who use denial and avoidance tend to be less compliant with care, while those with more hopefulness and “optimistic acceptance” are more adherent.

Among the coping strategies that Ms. Dvorak recommended are exercise, social support, communication with the CF team, therapy, goal setting, seeking help, the prevention of stressors, and involvement with the CF community. Harmful coping strategies include medicating one’s feelings with excessive drugs, the overuse of narcotics, noncompliance and poor self-care, dishonesty, unrealistic living standards or responsibilities, and passive aggressive behavior.

An effective social worker is able to identify which treatment option will help a person to mitigate stress and depression. Ms. Dvorak emphasized that psycho-



Meg Dvorak, L.C.S.W.

therapy is not failure – it can teach new ways of coping, and reduce stress. While therapy can be very helpful, not everyone needs this. Pulmonary rehabilitation, and activities such as yoga and meditation can all be effective. Religious participation and spirituality can be very helpful. In addition, social support networks, support groups and exercise programs have demonstrated positive results.

Dr. Birnbaum, the evening’s second speaker, discussed the challenges involved with the process of sorting out emotional from general medical struggles. As the physician overseeing the in-patient unit at Stanford Medical Center that treats people with significant psychiatric conditions, and also those with psychological issues related to physical health problems, Dr. Birnbaum emphasized how difficult it can be to arrive at a clear diagnosis.

According to Dr. Birnbaum, psychiatric symptoms may be “normal” for someone with significant medical issues. A patient’s experience while coping with his or her disease may present as a clinical event, and practitioners need to be careful to acknowledge what is being said “but not to over-pathologize it.”

While Dr. Birnbaum reiterated that those with CF do not have higher rates of psychiatric diagnoses, he reinforced that patients with CF may exhibit anxiety, apprehension,

(Continued on page 12)



CF Teen and Adult Retreat July 31st - August 7th, 2011 Vallombrosa Center, Menlo Park, CA



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Stem Cells (continued from page 1)

cell types. ASCs have been isolated from the blood and bone marrow, as well as hair follicles, skin, fat tissue, muscle, airways and lung, the central nervous system, and various other tissues.

Until recently the more controversial stem cells have been the embryonic stem cells (ESCs), which are derived from human embryos. These cells are of particular interest because they are pluripotent and have the potential of becoming any cell type within the body.

The controversy in regenerative medicine concerning the use of human embryos to generate ESC lines has limited their potential development as an effective therapy to regenerate tissues that have been damaged or destroyed as a function of disease pathology. Since ESCs are generally not available from the individual that requires treatment, there is also the issue of immune system matching, which is imperfect and requires a modulation of the immune system to prevent rejection of the allograft.

There have been attempts to overcome the issue of immuno-compatibility of ESCs through the development of patient-specific cell therapies using the nucleus from a patient's cells and performing somatic cell nuclear transfer (SCNT) into donated female oocytes (eggs) in which the nucleus has been removed. Unfortunately, SCNT in human oocytes is still beyond the present technology and is also fraught with controversy.

A recent breakthrough in regenerative medicine appears to have the potential of overcoming the dilemma we face in the U.S. regarding the therapeutic use of ESCs. This breakthrough involves the reprogramming of mature, differentiated somatic cells through the introduction and activation of cellular genes that can convert the somatic cells into cells that resemble ESCs. These induced pluripotent stem cells (iPSCs) have features that appear to be nearly identical to the ESCs, in that they are pluripotent and appear to be able to become any cell type in the

body. This is a significant advance in the ability to create patient-specific therapies, especially for individuals with CF.

It will, of course, be necessary to correct the genetic mutation in the cystic fibrosis transmembrane conductance regulator (CFTR), so that tissue identical to the patient's own, without the disease-causing mutation, can be used to engraft and repair the tissue that has been damaged.

While the studies carried out thus far have suggested that the iPSCs have the potential to generate a tissue of choice given the appropriate cell growth conditions, there is still significant work that must be done before this form of cellular therapy can be applied as a therapeutic intervention for CF, or any other inherited disease. However, given a concentrated effort by the biomedical research community and advocacy by patient groups for adequate funding support, this future vision can become a reality and change the lives of patients with CF.

Teens and Cystic Fibrosis: The Quest For Healthy Independence

By Siri Vaeth-Dunn

As anyone who has survived adolescence knows, it can be fraught with many challenges. Dramatic physical, intellectual and emotional changes occur simultaneously at a time when one's peer group may supersede parental influence. Teenagers seek increased independence from their parents, and experiment with how this independence will be manifested. While some of the choices made during this period can be detrimental to teens' health, for those with cystic fibrosis (CF), the consequences can be far more serious.

Developmentally, teenagers often feel invincible, and have a more difficult time connecting the impact of their current activities with their future. Adolescent experimentation can include many things: from new hair colors, body piercings and styles of dress, to drug and alcohol use, and sexual activity. Because the peer group often takes on greater importance and influence, many teens seek to fit in, and do not want to stand out as "different." It is "normal" for adolescents to challenge authority. These developmental issues face all teens, but their impact can be accentuated for those with CF.

Many teens with CF are consistent with their treatments, and in studies, these "adherent" teens said that doing so gives them more energy and enables them to take part in activities, and that when they had skipped treatments, they experienced negative impacts. Nonetheless, adolescence has been identified by numerous studies – and countless parents – as a challenging time for adherence to the CF medical regimen due to the developmental issues discussed above. In studies, teens cited numerous reasons for skipping treatments, including a desire to fit in and not lose time with their peer group, being frustrated with being told what to do, and



the sense that treatments are not having an impact.

Cystic fibrosis is a progressive disease, impacting numerous organs and systems in the body. Teenagers have spent much of their lives in the CF clinic, where the care team has communicated primarily with the parents. As teens transition to young adulthood, it is important that information is clearly communicated to them as well. There are often medical issues that teens may be hesitant to ask about in front of their parents, and it is important that they have the opportunity to speak alone with their team.

Members of the care team should understand that teenagers are often embarrassed to ask questions, and should present information proactively. Many teenage girls with CF suffer in embarrassed silence with uncomfortable yeast infections caused by the antibiotics they are taking. The nurse or physician should ask girls directly about this, to provide them the opportunity to allay their fear and shame, and to provide them with treatment. Additionally, many teens with CF - primarily girls - experience stress incontinence due to increased pressure

on the bladder from chronic coughing. There are exercises that can reduce the symptoms, but this is another issue they may be too shy to bring up on their own.

For many teens with CF, puberty is delayed, which can impact their self-esteem and accentuate their sense of being different. Reproductive health may be hard for teens to talk about, yet must be addressed. CF can impact birth control options for girls - for example, certain antibiotics can interfere with the effectiveness of oral contraceptives. And while male teens with CF are likely infertile, a slight percentage are not.

Teens need to be reminded about the importance of respiratory therapy. While scare tactics are seldom successful, teens who spend more time on their own need to feel comfortable communicating any increased respiratory symptoms. The CF medical team should make teens aware of complications such as hemoptysis (coughing up blood), which impacts over 60% of people with CF, so that they know what to do if this occurs. Nutrition is key for teens with CF, who due to malabsorption need extra calories, and often, extra calcium. For those teens with CF related diabetes (CFRD), the care team must help teenagers understand the direct correlation between blood sugar levels and lung function.

While healthy adolescents need over 9 hours of sleep each night, few teenagers achieve this, with one recent study finding that only 15% of surveyed teens slept over 8 hours per night during the school week. For teens with CF, the need for sleep is even more vital for optimal health, yet studies have found that they consistently report sleep disruptions.

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Teen Issues (Continued from page 9)

A chronic cough, and the side effects of medication can play a large role. In addition to causing a greater vulnerability to infection, the lack of sleep can also impact students' ability to concentrate, and can lead to dramatic mood swings. Because teens with CF are more prone to anxiety and depression, lack of sleep can worsen the situation.



Teens with CF report being depressed due to feeling physically unwell, the challenges of endless daily treatments, the side effects of medications, missing major life events (sports, parties, concerts, prom) due to frequent hospitalizations, and feeling different from their peers. As one teen put it, "Living with CF is like having a clock ticking above my head. My friends don't have to deal with that." In addition to issues specific to their disease, these teens also cope with the multitude of stressors that teenagers commonly face.

Among these stressors is peer pressure. Just because teens with cystic fibrosis have multiple health challenges does not mean that they are immune to the pressure to experiment with cigarettes, drugs, alcohol and sexual behavior. While studies find lower rates of "risky"

behavior with CF adolescents, a study of teens at the five major CF centers in North Carolina found that 1 out of 5 teens had smoked cigarettes, and nearly 30% had had intercourse. Another study found that youth with chronic health conditions had similar rates of sexual activity as their healthy peers.

Teens with CF may use alcohol or drugs to self medicate — due to depression and anxiety — seeking a temporary relief from the challenges of CF. Depression also contributes to self harm, namely "cutting," as well as eating disorders. Because self-care and adherence

to one's medical regimen is negatively correlated to levels of depression and anxiety, parents and the CF care team must help teenagers to find support and develop coping mechanisms. These can include therapy, sports, artistic expression, spirituality and participation in CF-related or other social support networks.

In many CF Centers, the teen years mark the transition from the pediatric to the adult CF Center. This can be a challenging time, as youth are often nervous about seeing a new care team. At the same time, many teenagers are thinking about goals beyond high school. The process of applying for colleges can be both exciting and daunting. For teens with CF, the prospect of moving away from all that is familiar, without the daily health care support provided by their parents, can create simultaneous excitement and anxiety. Teenagers should be encouraged to take on increased responsibility with their regimen to help them feel secure in their ability to manage on their own.

Teenagers with CF cope with extraordinary challenges. By acknowledging the unique pressures they face, and helping them to access the resources and support they need, teens will be better able to navigate their adolescent years. They must be recognized for their resilience, and supported in their quest for healthy independence.

Links For Teens

<http://www.cfvoice.com/index.jsp>

<http://www.cfliving.com/CLiving/>

<http://www.hopkinscf.org/teens/living/taking.html>

<http://www.cysticfibrosis.com/>

<http://kidshealth.org/teen/>

http://www.cysticfibrosis.ca/assets/files/pdf/Sexuality_and_CF_adolescentsE.pdf

<http://www.cfri.org/scholarship.shtml>

Anti-Inflammatory Regimens

(Continued from page 1)

there is increasing debate about their long-term effectiveness in reducing lung inflammation. The Cystic Fibrosis Foundation (CFF) now advises against the routine use of inhaled steroids in people with CF unless they also have asthma.

High-dose ibuprofen has long been recognized as an effective anti-inflammatory therapy. Clinical trials show that with proper dosing — often 2 to 3 times higher than that commonly prescribed — CF patients with mild lung disease showed less decline in pulmonary function, preserved body weight and fewer hospitalizations. While ibuprofen is one of the most cost-effective anti-inflammatory medications available, it is used by only 10% of CF patients in the U.S. The vital necessity of achieving a targeted peak plasma concentration, and the need for annual blood tests to confirm this peak, were cited by practitioners as deterrents to prescribing the therapy, as well as concerns over gastrointestinal complications and hemoptysis. It has been theorized that many CF disease symptoms are similar to those experienced with high-dose ibuprofen, and that the ibuprofen may not be to blame. This was supported by a two-year study in Canada, where more subjects taking the placebo dropped out due to complications that they attributed to the ibuprofen, than those actually taking the drug.

Azithromycin is now part of the standard protocol for CF treatment. Clinical trials show that those CF patients who take azithromycin three times per week have

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The CF Quality of Life Program: A Living Legacy of Peter Judge

By Ann Robinson

The Cystic Fibrosis Quality of Life Program: A Living Legacy of Peter Judge, is designed to address the emotional, psychological and spiritual impact on the physical well-being of those living with cystic fibrosis. Peter was the son of Tom and Julie Judge. He was born in 1962 and was diagnosed with cystic fibrosis (CF) when he was 17. That same year, he entered college, studying engineering at UCLA. Peter soon became depressed with anxiety related to his illness. After struggling with trauma, stress and loneliness, he became active in his church

by contributing money and serving meals to the poor. Peter died in 2005. The family's purpose in starting the Quality of Life Program is to complete Peter's journey.

The Quality of Life Program was developed by chronic disease specialists, CF professional caregivers, families and patients to treat the emotional impact of cystic fibrosis on CF patients and their various caregivers. The trauma caused by stress, depression, loneliness, poor self-image, grief, anger, addiction, and the difficulty of achieving independence is well known in the CF community. The program is a collaborative effort with the Institute of



Peter Judge

Transpersonal Psychology and is located in their Community Center for Health and

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The Heart of a Volunteer: Eric Marten

By Bridget Barnes

New to CFRI, but not to CF, Eric Marten began volunteering his film expertise last summer at the annual National CF Family Education Conference, where he conducted interviews with parents of children with CF. His work culminated in a promotional video with an original musical score by Alex Jenkins that is now featured on CFRI's website. Since then, Marten, who turned 35 in March and has CF himself, has been instrumental in bringing CFRI's programs about cystic fibrosis to people all over the world.



Eric Marten

"Carroll and I had many meetings about the CF Discovery Series™. We discussed how we could bring it to as many people as possible," Marten explained. "I run the computer that is streaming the video live over the internet. It is my job to make sure that the signal is clean and getting out to the world. I also run the chat room that is associated with the video stream; that way people can log in to chat while they are watching. I welcome everyone that joins in the conversation and make sure questions from the audience are answered by the speaker."

While Marten was born with CF, only in the last five years has he gotten involved with the CF community. "I started by talking to other people with CF online, through Myspace groups. I was going through hard times and I needed someone to talk to about how I was feeling," he explained. "I found the CF groups on Myspace to be very supportive and refreshing to talk to about what I was going through." Over time Marten made many CF friends and returned the favor of friendship by helping them through difficult times as well.

When asked what inspires him to volunteer, Marten replied, "I have been touched by so many people with CF, and their caregivers, that I wanted to be able to use my skills to reach out and bring us together as a community. Knowing that each time we do a CF Discovery Series™ broadcast we are reaching out to people, and bringing them together in a safe, supportive place where they can interact with each other while learning about various aspects of living with cystic fibrosis, is inspiring. I have witnessed people in the chat room making new friends and exchanging emails. People from all over the world have told us how thankful they are for the Discovery Series—that there is nothing like it where they live, or that they are too sick to attend in person. That is what inspires me to keep coming back."

In preparing to live broadcast the CF Discovery Series™, Marten oversees all the technical components needed to insure that the streaming goes smoothly. "I help set up the light, audio equipment, and video camera as well as run the computer that has the video streaming software. I

(Continued on page 14)

CFRI's Rose Garden Quilt



For the past year at CFRI, our office has been brightened by the Rose Garden Quilt. Stitched by Elyse Elconin-Goldberg, this beautiful hanging is a tribute to CFRI Mothers' Day Tea senders and incorporates squares sent in honor and in memory of their loved ones with cystic fibrosis. It has been on display at different CFRI events and attracted visitors, many of whom have had a part in its design.



Pictured (from left to right) Elyse Elconin-Goldberg, whose creative energy brought the quilt together; Ann McKirgen, CFRI volunteer; Elizabeth Mayer, co-founder of CFRI; Becca Roanhaus, CFRI supporter and tea sender.

CF and Emotional Health (Continued from page 7)



Justin Birnbaum, M.D.

depression, sadness and dejection, and have insomnia or anorexia. While a patient with CF may be struggling with sadness or despair, they still may not be clinically depressed. Conversely, practitioners must pay close attention to determine whether there is a secondary condition that will impact the patient's health outcomes, and be ready to treat a parallel psychological condition if it exists.

Dr. Birnbaum outlined the key symptoms of Major Depressive Disorder (MDD). These include a sad mood, sleep disturbance, interrupted appetite, fatigue/less energy, feelings of worthlessness and guilt, suicidal ideation and loss of interest in activities. While people with CF may experience many of these symptoms, it may not be a major depression. Dr. Birnbaum emphasized that prior to diagnosis of MDD, a person must have these symptoms for at least two weeks. The key marker of severe depression is "true anhedonia," a complete lack of interest in life's activities and the inability to gain pleasure from enjoyable experiences. In addition, guilt and shame may be experienced.

The origins of psychiatric illness were explained by Dr. Birnbaum, which encompass genetics, environment, exposure, substances and stress. Medications can also have a strong impact on mood. For example, Dr. Birnbaum addressed the effects of prednisone on CF patients, including depression, mood, sleep

and appetite disturbances and irritability. Studies show that as the dose increases, so do symptoms of depression.

Limited studies of psychiatric illness in the CF population demonstrate inconsistent findings regarding depression, eating disorders and anxiety. Younger children (7-10) may have increased anxiety, and emotional disturbances may increase with age. While eating disorders are present in the CF population, there is no evidence of increased rates of anorexia or bulimia. Notably, parents of CF patients consistently report having increased rates of depression, anxiety and stress. Conversely, these parents rate their quality of life to be higher.

The emotional health of those with cystic fibrosis is vital to their physical health. Patients, parents and practitioners must pay close attention to this issue to ensure that emotional difficulties are identified and strategies are in place to help the person with CF cope with the multi-faceted challenges of the disease.

Mothers' Day Tea Still Blooming!

By Bridget Barnes

Once again we would like to offer a big bouquet of gratitude to our Mothers' Day Tea Senders! As of May, \$55,000 critical dollars have been raised to support CFRI's Elizabeth Nash Memorial Fellowship Program, as well as the annual National CF Family Education Conference, the Teen/Adult Day Retreat, and outreach materials such as *CF in the Classroom*, the *CF Website Guide*, and *CFRInews*. We are so very grateful to those of you who have participated in our biggest fundraising event of the year. Your generosity and support, which mean so much in light of the current economic climate, allow us to continue to fund



Tea senders: Norma Stuckert; Sallie and John Best

important research and provide current information to those with cystic fibrosis and their families.

Our goal this year is to raise \$247,000. We received our first donation of 2011 in February. Since then, over 1,434 donations have been received, with more arriving daily.

Barbara and Jim Curry, grandparents to Cameron, age 17 with CF, graciously hosted the Mothers' Day Tea "kickoff" reception at their home on March 20. Patricia Moylan, (a longtime friend of Tara Weir, who passed away in 1996 from CF) won the raffle and took home a lovely pastel painted teapot donated by longtime CFRI volunteer Pat Flynn.

If you still would like to become a Tea Sender, it is not too late! For complete packets including invitations, envelopes, tea bags and mailing labels, please call our office at 650.404.9975, or email: CFRI@cfri.org, or order online at <http://www.cfri.org/formMDT2011.shtml>. We will be happy to provide you with everything you need. Thank you!

CFRI Mothers' Day Tea is celebrated every day of the year to raise vital funds for CF research and programs!

Now, you can create your own personal page to email family members and friends who will support the cause. Invite them to have a *Virtual Cup of Tea* with you and to make a donation to help you reach your goal.

CFRI's Mothers' Day Tea began more than 35 years ago, when a small group of mothers who were committed to raising funds for cystic fibrosis research, asked their family members and friends to share a cup of tea with them across the country. Help us grow what they started!

Visit <http://www.cfri.org/formMDT2011.shtml> or call 650.404.9975.

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Reports
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(Sample Page)
Have a Cup of Tea
for CF Research and Programs!


- High Tea (\$1,000.00 +)
- Tea Pot (\$500.00 +)
- Tea Cozy (\$250.00 +)
- Tea Cup (\$100.00 +)
- Tea Crumpet (\$50.00 +)
- Tea Cake (\$25.00 +)
- Tea Cookies (\$10.00 +)

Enter Donation Amount Here:
*Currency shown: US Dollar
 \$:

I want to enter the donation in my own name: pre-fill the entry form for me.

My Fundraising Goal: **\$250.00**

Money Raised to Date: **\$0.00**



Jane with her teapot and teacup.

Dear family and friends,
 Please join us for a virtual cup of tea to help raise funds for cystic fibrosis research and programs. Your support means so much. Thank you!

“In Honor of” and “In Memory of”

Our “In Honor of” and “In Memory of” page provide the opportunity to honor a person, family, or special event or remember a loved one who was lost.

If you wish 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.

Mail your contributions to:
CFRI, 2672 Bayshore Parkway, Suite 520,
Mountain View, CA 94043

Contributions listed here were received
from January 15 - April 15, 2011.

In Honor of

Haleigh Baker	Joseph Fraker	Carly McReynolds
John and Bridget Barnes	Cody Franks	Anna Modlin
Lucy Barnes	Franks’ Walk	Jessica Nett
Joseph Batchelder	Emily Fredrick	Jereme Pierce
Maggie-Faye Bendz	Jessica Fredrick	Theresa Plesha
Alison Best	Jenise Giuliano	Melissa Predny
Wendy Davis Bosarge	Larissa Giuliano	Bune Primack
Anita Cass	Jennifer Goodwin	Robyn Primack
Lauren Colonna	Barbara Greenberg	Rebecca Roanhaus
Mary Convento	Bonnie Grossman	Matthew S. Ruckersfeldt
The Curt Cournane	Sonya Haggett	Jerry Sinnaeve
Family	Barbara Harwood	Hal Soloff
Barbara Curry	Susan Hoffman	Matt Spadia
Kevin Dilworth	Peggy B. Jones	The Tripp Family
Tess Dunn	Kaeti Pierce Lillibridge	Michael Matson Vitousek
Ryan Foster	Larissa Marocco	Devin Wakefield
Jacob Fraker	Mikayla McDonald	Kassi Watkins
		Lauren Williams

In Memory of

Agnes Caleri	Doug Fenell	Ken Kusalo	Eva Petersen-Stephens	Mel Stich
Alana Connick	Douglas Graham	Dawn Longero	Anthony G. Pierce	David Stuckert
Kenneth D. Beaton	Linda J. Halunen	Rusty McAlpin	Bonnie Predny	Laurie Stuckert
Dolores Belew	Virgil Hanson	Elizabeth Nash	Tom Rivers	Evelyn Suerth
Anne Beltrame	Te Harrel	Kim Nelson	Bruce Royer	James Tennyson, Sr.
Marilyn Capwell	Stephanie Huff	Scott Nelson	Randy Rupracht	John Trask
June DeCosta	Kevin Keaveney	Ashley Nowlin	Dhea Schalles	Louis Anthony Trigueiro
Jordan Dell’Era	Lori Kipp	Diane Pacini	Tim Schenck	Howard Weintraub
Sylvia Duffy	Ann Kistner	Phyllis Partridge	Joseph Marden Sinnaeve	Tara Weir
Mrs. Edmunds	Edmond Klein	Mr. Peters	Tammy Smerber	Richard Wheelless
			Helen Steinke	Thelma Dickey Worthen

Eric Marten (Continued from page 11)

am in charge of setting up the show and broadcasting and recording it live over the internet. I also create events on facebook to let people know when the next Discovery Series is taking place.” He confesses, “It’s stressful at times because technology is never perfect, so sometimes we have issues getting all the moving parts to work together, but we are getting there. It’s fun when everything is running smoothly and people in the chat room are joking around with each other.” Marten is currently working on an animation piece to open the live broadcast involving the CFRI/live! logo and other graphics.

As for CFRI’s digital future, Marten envisions a website that is dedicated solely to the Discovery Series. “It would have an archive of all the videos we have produced so far; would stream future live broadcasts, including chat rooms; and would include a facebook-like profile system so that people living with CF could create a profile and communicate with other people. I would like to see all the educational materials that go along with the presentations posted online as well,” he explains.

“I get a sense of pride that I am helping bring people with cystic fibrosis together

who wouldn’t normally be able to,” Marten says humbly. “It is worth all the trouble of putting on one presentation of the CF Discovery Series™, as long as one person says they love the fact that we put it online and that they learned something, or had a good time chatting with people, and know that they are not alone.”

We are grateful to Eric Marten for his worldwide commitment to CFRI. His technical expertise and inspiring vision help CFRI broaden its impact immeasurably by broadcasting our very reason for being into every corner of the world. Thank you Eric!

Peter Judge (Continued from page 11)

Wellness in Palo Alto, California. Due to remarkable improvements in CF treatments, those with CF are living longer, healthier lives. The compassionate and professional counselors at the Quality of Life Program at the Community Center for Health and Wellness have been trained to understand and address the other half of patient care: the impact of chronic illness on emotional health and quality of life. The program functions under Stanford University's 501(c)3 nonprofit fiscal sponsorship and has received

programmatic guidance from Cystic Fibrosis Research, Incorporated, which assisted in its planning and development.

Due to licensing restrictions, this program is currently limited to those living in California. Professional fees for counseling have been reduced to \$40 per hour, but no one is turned away for lack of funds. Safety protocols to prevent cross infection between CF patients have been implemented. CF patients seeking in-person services must

submit a sputum culture prior to acceptance in the program. If a CF patient has a sputum culture that prohibits face-to-face contact with other CF adults, that person can still participate via telephone or Skype.

For further information or to schedule an appointment, please contact: The Institute of Transpersonal Psychology, Community Center for Health and Wellness website at <http://www.itp.edu/counseling> or by phone at 650.855.8898.

Anti-Inflammatory Regimens (Continued from page 10)

better lung function, weight gain and quality of life. Studies indicate that azithromycin has both an anti-infective and anti-inflammatory impact, as it seems to inhibit neutrophil recruitment and "oxidative burst," the release of chemicals from the neutrophils which can further damage the airway tissue. While the benefits of azithromycin are clear, there is concern about increased antibiotic resistance due to its long-term use.

Fortunately, there are new anti-inflammatory therapies available that show great promise. Most notable is high-dose oral N-acetylcysteine (NAC), an antioxidant glutathione prodrug that is a precursor to glutathione (GSH). Researchers at Lucile Packard Children's Hospital at Stanford and the Institute of Metabolic Disease at Baylor University found that in addition to extraordinarily high numbers of neutrophils in their airways, those with CF had significantly lower levels of basal cellular GSH, a pivotal antioxidant.

It is believed that the low level of antioxidants impacts the quantity of neutrophils, as well as their rate of degranulation and death. Researchers sought a means to increase the level of GSH in the peripheral blood neutrophils that home in to the airways before they made their way to the lungs. When these neutrophils with elevated GSH levels reached the airways, they produced a more controlled inflammatory response to the bacteria and milieu of the CF airways.

In a clinical trial of people who took NAC three times daily, it was found that

short-term, high-dose oral NAC "significantly decreases neutrophil count in CF airways." Study results also showed that this therapy "significantly decreases CF airway elastase activity, the best predictor of CF airway dysfunction." The correlation between high neutrophil counts and lower lung function has been clearly established, and the addition of NAC to the CF treatment arsenal is encouraging.

Dr. Carol Conrad, Assistant CF Center Director, and Director of the Pediatric Lung and Heart-Lung Transplant Program at Stanford, is recognized as one of the leading researchers in NAC therapy. Says Dr. Conrad, "In addition to the potential longer term preservation of lung function effect of NAC, multiple other possible benefits have been discovered that can be quite beneficial to CF patients. NAC can also decrease inflammation in the gut, improving absorption of essential nutrients. There were no detected toxicities from the NAC formulation used in our study."

Dr. Conrad cautions that patients with CF who use NAC should obtain the specific formulation that was tested in her study to ensure proper dosing and prevent its degradation. While NAC and GSH are available in various formulations in health food stores, these products have not been tested and are often degraded by packaging processes to unusable and toxic forms, and patients are highly discouraged from using them. Adds Dr. Conrad, "There are no published studies that have demonstrated any benefit to use of oral or inhaled GSH."

According to the CFF's website, several anti-inflammatory therapies are currently being studied. They include a pilot study in which infants with CF who were diagnosed through newborn screening are fed a formula that includes a fish-oil fatty acid known as DHA, believed to be a key anti-inflammatory. Another trial involves KB001, "a humanized monoclonal Fab fragment" that will be developed as an anti-inflammatory therapy for CF patients chronically infected with *P. aeruginosa*. Researchers at the University of New Mexico are studying whether sildenafil (the drug most commonly known as Viagra) can lower airway inflammation as well as measures of airway infection in CF patients. In addition, GSK SB 656933, a once daily oral anti-inflammatory agent has entered phase 2 trials. And in England, phase 2 trials recently concluded that studied VR496, a dry powder formulation that has received orphan drug status in both the U.S. and Europe. After four weeks of treatment with VR496, induced sputum samples showed decreased levels of neutrophil elastase and total cell counts when compared to those taking a placebo.

Anti-inflammatory therapies represent a critical piece in the CF treatment regimen puzzle. The damaging role that chronic inflammation plays in the destruction of airway tissue is well documented. With effective therapies, this inflammation can be lessened, leading to improved health outcomes for those with cystic fibrosis.



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CFRI's Mission

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

CFRI's 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.



Get to know us:

www.CFRI.org 650.404.9975

