cfri community
44 Years of Research, Education & Support
Spring 2019

Editor's Note: In October 2018, CFRI hosted an Externally-Led Patient-Focused Drug Development Meeting on Cystic Fibrosis, to bring the voices and experiences of those living with CF to representatives of the Food and Drug Administration. During this extremely impactful meeting, the painful realities of daily living with CF and the urgent need for new therapies were expressed. The summary Voice of the CF Patient Report can be read in its entirety at cfri.org. The following presentation by CF advocate Emily Kramer-Golinkoff provided moving testimony to the work still yet to be done.

Joy

By Emily Kramer-Golinkoff, MBE

Tuesday May 29th was a gorgeous day. Seventy degrees, blue sky, sun kissing my skin, one of those days where the universe was bursting with potential and it was hard not to feel content. I remember sitting outside the Perelman Center for Advanced Medicine when I got a call that changed everything. "Emily," my doctor said with uncharacteristic alarm, "Your x-ray revealed that your lung is partially collapsed in three places." I remember hanging up the phone, looking up at the big blue sky, and then back down at my body, stunned that a body so seemingly functional could be so secretly broken.

Meeting with my doctor, I learned that there were two treatment options for lung collapse, officially called pneumothorax, a life-threatening complication of advanced lung disease. The first was to closely monitor via x-ray and CT to see if time and rest could resolve the collapse. The alternative was a procedure called pleurodesis that involved adhering the lung to the chest wall. The challenge is that it presents complications for transplant, an important consideration for someone with advanced stage disease like me. My hope was to do everything possible to avoid pleurodesis.

Ironically, treatment for lung collapse is antithetical to the standard of CF care in profound ways. Mainly no airway clearance and no exercise.

You can imagine the tailspin that throws your head into. The very strategies to which you devote four hours every single day, the ones that you depend on to control the infections ravaging your lungs, those are the very therapies that you must withhold so that your collapse might naturally heal. And therein lies the real predicament. It becomes a race. Can you go enough time without airway clearance for your lungs to re-inflate before an infection rages out of

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Emily Kramer-Golinkoff

Bacterial Community-Level Susceptibility Testing for CF Lung Infections

Lucas Hoffman, MD, PhD | University of Washington, Seattle

Chronic lung infections of people with cystic fibrosis (CF) do not respond to antibiotics as predicted by current concepts and culture methods. In general, antibiotics are chosen for treating CF patients based on the *in vitro* susceptibilities of a few isolates of the culturable "traditional pathogens" from their respiratory secretions. However, research demonstrates these methods do not result in the selection of antibiotics that reliably lead to clinical improvement. In addition, researchers have been unable to establish significant, patient-level relationships between changes in standard CF respiratory culture results and clinical response to treatments. Therefore, the microbial determinants of clinical response to antibiotics are not clear, making it difficult to know how to improve our treatment approaches.

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Joy *Continued from front cover*

control? Lung collapse or infection, which is your poison?

And so on that beautiful day, I set off on my race against infection. My doctor sent me home on periodic high flow oxygen therapy to re-inflate my lung, my first time ever on supplemental oxygen, and he gave me strict instructions to come into the hospital for admission ASAP if I felt even a slight infection. After a lifetime of treatments, "time, rest, and no airway clearance" felt like an evil prescription of omission.

I knew enough about lung collapse to know that it was the next step to end-stage disease and I felt desperate for information. I plunged into the online CF community. I read story after story of people who experienced lung collapses and permanently lost huge chunks of lung function, who were unexpectedly thrust into the terrifying world of transplant, or even worse, for whom the treatment for lung collapse questioned their future of transplant altogether.

I read of people who had pleurodesis for whom it proved unsuccessful; people who told me they were never the same again, for whom that first lung collapse was just the start of many more to come. And I read, time and time again, that the pain of pleurodesis was utterly excruciating.

I'm a resilient, hopeful person. All I was looking for was the tiniest glimmer of hope, of possibility that it could be okay. But the deeper I probed, all I found were stories of catastrophe or, at best, slow demise. I couldn't find even one story with a positive ending.

The more I learned, the longer the list of restrictions grew. No airway clearance, no exercise, no spirometry, no lifting. And as horrific as they were, I felt I could endure. A lifetime with a disease with a penchant for rearing its head at inopportune times had taught me how to get brutally knocked down and stand back up again. And yet, the one thing that made me question whether or not I could endure was the one about joy.

Learning that flying was off limits not only immediately following lung collapse but that it would forever be a major trigger, that was the one that threatened to crush my soul. Even the most unimaginable pain and suffering are endurable with meaningful punctuations of joy. For me, that joy is traveling to faraway places. The thought that this disease could not only rob my function and my future, but that it could also take my single greatest joy - that was the part that wrecked my heart, that made me question, for the first time in my life, if I could indeed endure.

CF, you take my breath, you take my friends, you take my dreams of a career and a family. You take my hope, my promise, my very potential. You take my future, and now, you take my joy.

Emily co-founded Emily's Entourage, a 501(c)3 that accelerates research and drug development for nonsense mutations of CF. To learn more, go to emilysentourage.org, where you will also find their social media links.

Emily Kramer-Golinkoff with her mother – and Emily's Entourage cofounder – Liza Kamer, MSW.

Move-a-Palooza!

September 14 - 21, 2019

Moving toward a cure!

Join Your Peers Nationwide: Raise Cystic Fibrosis Awareness & Support the Search for a Cure!

Pick your favorite way to move: reps in a gym, running, hula hooping, swimming – you decide! Ask your friends and family to sponsor you. CFRI will provide a link to our online fundraising platform, where your supporters can make donations. Choose your day - anytime between September 14 and September 21. Take pictures and/or video and post on social media #CFRI-Moveapalooza. CFRI suggests a $100 minimum goal per participant, but there's no required amount, and no penalty if you don't reach your goal. Those who raise a minimum of $100 will receive a Move-a-Palooza t-shirt!

Questions? Email Tony Adessa at tadessa@cfri.org or call 650.665-7586.