A Multi-'Omic Approach

Continued from Front Cover

or disease status, the role of the respiratory microbiota in diseases of the airways is still poorly understood. Recent studies of the lung microbiota of cystic fibrosis (CF) patients have shown that a diverse microbiota colonizes the CF lung during childhood and that the composition of the lung microbiome changes, and diversity decreases, as the disease progresses. The upper airways are also affected by the defect in the CFTR gene (causing CF disease), and because of this, CF patients commonly present with chronic rhinosinusitis (CRS).

Previous studies of CF patients with CRS have shown that the upper airways are colonized by identical strains of a major CF-pathogen, *Pseudomonas aeruginosa*. A recent study of the upper and lower airway microbiome in CF-CRS patients using marker gene sequencing demonstrated a high degree of similarity in the airway microbiome of CF-CRS patients, but strain-level resolution could not be achieved. These findings suggest the possibility of the sinus as the portal of entry for bacterial pathogens and other microbes into the lungs. However, it is still unclear how sinus microbiota affects the lung microbiota composition and lung inflammation. In our studies, we use a multi-'omics approach to robustly characterize the sinus and lung microbiota and determine the functional attributes of microbes that correlate with inflammation in the upper and lower airways.

Our research team has made good progress on the metagenomics analysis and we have demonstrated a unique bacterial and fungal signature in the upper and lower respiratory tracts of study participants with diagnosed CF and CRS. These studies will help to determine which microbes or their functions contribute to CF disease progression and will help direct future studies to define candidate microbes for which to develop targeted microbiome-based therapeutics against. Our study will help uncover the role of the upper and lower airway microbiome in CF patients and lead to better clinical management of this devastating disease.

CFRI's 33rd National Cystic Fibrosis Education Conference – *Learning, Laughing, and Loving with CF*

Continued from page 5

doctor, times got both better and worse, depending on the day. Middle age followed, because despite her doctor's somber observation, "You should be dead," she continued to wake up day after day assuming the role of Mommy, partner and CF volunteer. This brings us to present day, where the magic of Trikafta has completely changed the game, the role of Drill Sergeant has been dropped, and life is simply lived and enjoyed. Julie hopes to both entertain and motivate with stories from the trenches of identity building and loss, family disruption and generation, mental and physical health challenges and the lessons they taught, and how the tools of exercise, meditation, humor and love has sustained a life that could not be more full. *https://tinyurl.com/y2xs9omj*

Advocacy During the Pandemic

COVID-19 has had a devastating impact upon our cystic fibrosis community on many levels. While all 2020 in-person events were cancelled, CFRI has continued to raise awareness and advocate on behalf of the CF and rare disease community through virtual meetings with elected representatives, action alerts on pressing legislative issues, and informational updates on policies and legislation that impact our community.

CFRI has advocated independently and in partnership with other health-related organizations at the state and federal level to address discriminatory medical rationing - urging the removal of language from state emergency plans that excludes or deprioritizes those with advanced lung disease from receiving ventilators during a shortage. We have urged our community members to express their opinions related to access to telemedicine, and expansion of paid medical leave.

CFRI endorsed California Proposition 14 – Californians for Stem Cell Research, Treatments and Cures – so as to continue the California Institute for Regenerative Medicine, which has funded extraordinarily promising research for the CF community.

CFRI is raising awareness to halt the rapid expansion of Co-Pay Accumulator Programs, which no longer allow patients to apply their co-pay cards from drug manufacturers toward their annual deductible. Once patients exhaust their co-pay coupons, they discover that they still have hundreds or thousands of dollars due in out-of-pocket payments before their insurance coverage applies. Four states have outlawed these programs and federal legislation has been introduced to allow co-pay accumulator programs only when a generic drug equivalent is available. We will keep you informed.

CFRI remains firmly opposed to the use of the Quality Adjusted Life Year (QALY) to measure drug value and price. The use of QALY originated as a means to ration care, and it is inherently discriminatory toward people with chronic disease and disabilities. The use of QALY to assess medication pricing for the CF and rare community is unethical, subjective, and likely a violation of the Americans with Disabilities Act.

CFRI will continue to keep our community informed, supported and engaged as we cope with the challenges of COVID-19 and life with a challenging rare disease.