

## Trends in Nutrition

A conversation with Virginia Stallings, MD from Children's Hospital of Philadelphia (CHOP) and lead investigator for Encala's NIH funded trial (3-part series)

**EH:** *Dr Stallings, why did you choose cystic fibrosis (CF) as the lead disease state to study Encala?*

**VS:** The inventor of the structured lipid technology that makes Encala so well absorbed and effective was inspired to do this work to help with a young family friend with CF. He was committed to improving the lives of people with CF with better nutrition therapy to improve fat absorption and maintain a healthy weight. My ongoing work in CF and nutrition was a perfect match. Dr Yesair and I designed the NIH funded CF study and worked together on our large clinical trial. The successful results with better fat absorption, growth and nutritional status led to Encala now being used for clinical care.

**EH:** *What is the size of the opportunity in CF and how have the CFTR modulator drugs affected the opportunity?*

**VS:** The number of patients with CF is a little over 31,000 in the US with another 40,000 worldwide, and about 85% of all patients have significant GI/pancreas disease resulting in exocrine pancreatic insufficiency (EPI) and fat malabsorption. EPI is treated with the medication pancreatic enzyme replacement therapy or PERT. However, even with PERT about 25% of our adult and pediatric patients have ongoing fat malabsorption and GI symptoms and are unable to gain and maintain a healthy weight. Studies show that achieving the recommended weight and BMI is associated with better lung function and quality of life. Encala can help improve GI symptoms while supporting the essential growth in children and healthy weight in adults with CF.

The launch of CFTR modulators was a major advance in CF care to improve lung function and extend life. The lung improvement is related to the specific patient CF mutations and drug matching, and some had large improvements and some less. In the same pattern, modulators increased weight and healthy BMI in some patients. From a clinical nutrition view, however, many patients will require ongoing nutritional care including a product with Encala's clinically proven efficacy. According to the CF Foundation Patient Registry 2020 Annual Report, about 48% required tube feeding or enteral support. The Report documents persistent CF-related undernutrition: about 50% of adults have low BMI and growth faltering is common in the youngest patients.

**EH:** *Are there other areas in clinical nutrition where Encala would address unmet needs?*

**VS:** Yes, there are many patient populations much larger than CF where fat malabsorption and related GI symptoms and malnutrition are major clinical concerns. Encala can be beneficial in pancreatic or liver diseases where decreased lipase or decreased bile acids reduce efficient absorption of dietary fat. These conditions include chronic pancreatitis, pancreatic cancer, celiac disease, inflammatory bowel disease-Crohn's and ulcerative colitis, chronic diarrhea, irritable bowel syndrome (IBS), short bowel syndrome, and moderate to severe liver disease. The NIH estimates the prevalence of GI disease in the US is 60 – 70 million people.

**EH:** *Why is nutrition and measures like weight and BMI important for patients with these diseases and conditions?*

**VS:** Decades ago, patients were treated with therapies available for the specific disease with less awareness of the importance of nutrition in the treatment paradigm. This has changed over the years, as evidence is published showing the benefit of optimized nutrition status in patient outcomes. The 2018 Lancet Diabetes Endocrinology paper concluded that being underweight, a BMI of 18.5 or less, was associated with reduced life expectancy of more than 4 years in both women and men across every major disease category of cause of death except for travel-related accidents. Today, clinical dietitians and nutritionists are key members of treatment teams for many patients with chronic disease, not only GI diseases but also in oncology, transplant, renal and heart failure.