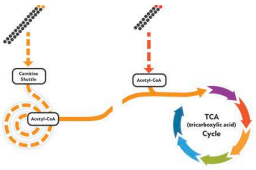
EXPERT PERSPECTIVES:

BRIDGING GAPS, OPTIMIZING TREATMENT IN ADULTS WITH LC-FAODS



Dear Colleague:

Thank you for your recent participation in the CE activity “Bridging Gaps, Optimizing Treatment in Patients with LC-FAODs” with Drs. Sandra van Calcar and Jerry Vockley and developed by the Annenberg Center for Health Sciences. As you continue to advance the care you provide to these patients, here are the key concepts for you to consider:

- LC-FAODs are hereditary metabolic diseases that impede the breakdown of long-chain fatty acids owing to enzyme deficiencies in the carnitine shuttle or β-oxidation pathway, affecting energy generation, especially during fasting or stress.
- Patients with disrupted fatty acid oxidation have energy deficits that may appear as hypoglycemia, muscular weakness, cardiomyopathy, and rhabdomyolysis, especially under metabolic stress.
- Treatment has shifted from traditional nutritional and symptomatic management with medium-chain triglycerides to more targeted therapies, including the FDA-approved triheptanoin, which bypasses metabolic blockages and provides an alternative energy source.
- The target daily dose of triheptanoin is 35% of total daily calorie intake, divided into at least four doses and administered at mealtimes or with snacks every three to four hours. The initial dose is based on whether the patient is new to therapy or switching from other medium-chain triglyceride products.
- Gastrointestinal adverse events (GI AEs) are common. Strategies to manage GI AEs include: 1) starting at a low total daily dose and titrating upward slowly; 2) administering in four or more small doses; 3) mixing with food or liquids; and 4) temporarily reducing the dose until symptoms improve.

We invite you to participate in other accredited activities we offer (www.Annenberg.net). Thank you.

Regards,

The Annenberg Center Team