



# The Annenberg Academy for Team-Based Care

## Interprofessional Care of Patients with Long-Chain Fatty Acid Oxidation Disorders

### PATIENTS WITH LONG-CHAIN FATTY ACID OXIDATION DISORDERS: KEY CONCEPTS

Long-chain fatty acid oxidation disorders (LC-FAODs) are rare metabolic conditions caused by defects in the genes that encode the enzymes required for fatty acid oxidation. These defects lead to a hindrance of the conversion of stored fats into energy during times of fasting or high-energy demands, such as exercise or illness. The end result is both an energy deficit and the accumulation of toxic intermediates that can lead to organ dysfunction. Treatment has historically consisted of diet modification and nutritional supplementation with even-carbon medium-chain triglyceride (MCT) that bypasses the defective enzymes in LC-FAODs. Although MCT supplementation is beneficial, patients often are unable to tolerate the high doses needed. Triheptanoin is an odd-carbon MCT approved as an alternative treatment option. Unlike even-carbon MCTs, the breakdown of triheptanoin results in the production of by-products that enter the tricarboxylic acid cycle at multiple points, thereby producing energy more efficiently. This allows for improved ATP production and gluconeogenesis, protein synthesis, and more efficient energy production.

Key concepts to keep in mind when managing patients with LC-FAODs are:

- LC-FAODs are hereditary metabolic diseases that impede the breakdown of long-chain fatty acids owing to enzyme deficiencies in the carnitine shuttle or  $\beta$ -oxidation pathway, affecting energy generation, especially during fasting or stress.<sup>1,2</sup>
- Patients with disrupted fatty acid oxidation have energy deficits that may appear as hypoglycemia, muscular weakness, cardiomyopathy, and rhabdomyolysis, especially under metabolic stress.<sup>2-4</sup>
- Treatment has shifted from traditional symptomatic and nutritional management with even-carbon MCTs to more targeted therapies, including the FDA-approved triheptanoin, which bypasses metabolic blockages and provides an alternative energy source.<sup>2,4,5</sup>
- 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 MCT products.<sup>6-9</sup>

- New patient: Start at approximately 10% of the total caloric intake divided into 4 doses per day. Increase the dose by approximately 5% every 2 to 3 days until reaching the target dose
- Switching from other MCT products: Discontinue the previous MCT product and initiate triheptanoin at the last tolerated daily dosage of the MCT divided into 4 doses per day. Increase the dose by 5% every 2 to 3 days until reaching the target dose.
- Gastrointestinal adverse events (GI AEs) are common with triheptanoin. 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 per day; 3) mixing with food or liquids; and 4) temporarily reducing the dose until symptoms improve.<sup>6-9</sup>

### INTERPROFESSIONAL COLLABORATION PRINCIPLES

For the evolution in treatment to be of optimal benefit to patients, comprehensive strategies for choosing, delivering, monitoring, and modifying therapy have become especially important. As a consequence, care is typically provided by an interprofessional, multidisciplinary care team that extends beyond physicians to include nurses, nurse practitioners, pharmacists, physician associates, social workers, and others, often involving collaboration between providers in community and academic settings.

To that end, the following reflects a conversation among 3 healthcare professionals about interprofessional care and how collaborative practices and teams can strengthen our health systems.

**Christopher Flores, MD:** In medical school, I was taught that the patient-doctor relationship was the most critical and important dynamic in healthcare. But after 30-plus years in clinical care, I can attest that healthcare is a team sport and we deliver care in teams of individuals with different training, different skills, different talents. And we teach each other, we learn from each other, we brainstorm and solve problems to meet the needs of the patient.

I want to make a point that interprofessional refers to clinicians in different professions, such as nurses, nurse practitioners, pharmacists, physicians, and physician associates. Multidisciplinary refers to clinicians in different specialties or sub-specialties, such as cardiology,



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dermatology, and oncology. LaTosha, do you want to talk about interprofessional collaboration?

**LaTosha Mollette, DNP:** The World Health Organization defines interprofessional collaboration as when multiple healthcare workers from various backgrounds work together with patients, families, and communities to provide the best healthcare possible.<sup>10</sup> This is exactly what teamwork should look like, but it is important to remember that healthcare teams can vary from patient to patient.

I work in a rural setting, working together with various healthcare professionals to improve access to needed healthcare services, which helps to prevent unnecessary delays in care and treatment. Ultimately, working together as a team helps to meet the needs of others to improve health outcomes, patient care, and safety.

**Christopher Flores, MD:** I think all of us in healthcare are trying to figure out how to do things better, make life easier for ourselves, and make everybody happier, patients, our staff, and ourselves, to make things more sustainable. Interprofessional collaboration can accomplish this. There's a growing body of literature that shows that interprofessional collaboration can improve patient outcomes, acceptance of treatment, and satisfaction.<sup>11-14</sup> It can decrease costs, improve efficiency, reduce disparities, improve health equity, and make things more sustainable for providers.<sup>11,15-22</sup>

The Interprofessional Education Collaborative has identified 4 behavioral competencies for effective interprofessional collaboration: 1) roles and responsibilities; 2) values and ethics; 3) communication; and 4) teams and teamwork.<sup>23</sup> Michael, do you want to talk a little bit about the roles and responsibilities?

**Michael Smith, PharmD:** Certainly, it's one of these things that we don't often think about, but it's important for 2 reasons. One, understanding our own responsibility and roles that we have within our team and what our teammates can expect from us in terms of what we can deliver to them and deliver to patients.<sup>23</sup> The other is understanding what your team can do for you as well, so that you understand their educational background and you can help them practice at the top of their license by utilizing their skillset to the fullest extent.

It is increasingly common for clinicians, even those within a profession, to take different educational and practice paths leading to clinical practice. As an example, there are various differences in training a pharmacist. Nowadays, all pharmacists graduate with a Doctor of Pharmacy degree (PharmD), but some of us have done 1 or 2 years of residency training or postdoc fellowships. So, getting to know your teammates and what you can expect from them and what they can expect from you can really help your team function at a high level.

**Christopher Flores, MD:** We talk about values and ethics as well.<sup>23</sup> In medicine, we're constantly required to make very difficult and complicated treatment decisions for our patients. We really are missing a great opportunity if we don't involve the opinions of all the members of our team. What does the nurse think about this plan or what does the social worker who has talked to the family members think about this plan? Or what does the physical therapist think, who has been working with the patient for the last 3 days? LaTosha, what do you think about communication?

**LaTosha Mollette, DNP:** Communication is essential in everything we do, and it's how we're able to effectively achieve goals, as well as improve relationships and interactions with others.<sup>23</sup> The healthcare system is often described as being fragmented with little communication and collaboration, but when healthcare professionals communicate responsibly and respectfully, this allows them to overcome differences and work together to accomplish a shared goal, including learning from each other, to better improve patient outcomes and safety.<sup>16,24-26</sup>

**Christopher Flores, MD:** We're talking a lot about teams and teamwork. Michael, do you have any other points you want to make about teamwork?

**Michael Smith, PharmD:** Most of us are members of various teams during the course of a typical day. Think about the team members that you work with to take care of patients, but also think about a team from a networking standpoint. Do you have a network of like colleagues? As an example, other pharmacists in our healthcare system may reach out to me for advice about a patient with pain, whether or not I'm actually seeing the patient. We can make our team small, we can make them big, but we should be making our teams in ways that everybody's functioning at a high level and putting the patient at the center of the team.



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**Christopher Flores, MD:** Michael, LaTosha talked about the fragmented healthcare system and how interprofessional collaboration can help with that. Do you have any examples from your experience?

**Michael Smith, PharmD:** Think about the patient's experience through our healthcare system. Even if a patient receives all of their healthcare within 1 system, they often have to travel to many different places just to access care. From a primary care clinic to a hospital, to a specialty clinic, to a pharmacy. We can fill these gaps by using our interprofessional framework, our education, and allowing our collaborative practice teammates to step in and fill that.

**Christopher Flores, MD:** In conclusion, I just want to summarize that medicine is a team sport and that there is a growing body of evidence that supports the various benefits of interprofessional collaboration. LaTosha, Michael, do you have any final thoughts?

**LaTosha Mollette, DNP:** I think just being willing to change is crucial. I think sometimes we have become complacent in clinical practice, but our healthcare system is ever evolving.

So, learning how to be a team player always benefits everyone involved.

**Michael Smith, PharmD:** I've learned a great deal from my interprofessional colleagues, and I hope that I've helped them learn as well, with the ultimate goal of really improving patient care.

### INTERPROFESSIONAL CARE OF PATIENTS WITH LC-FAODS

The care of patients with LC-FAODs by an interprofessional, multidisciplinary care team is essential due to the complexity and multisystem impact of these rare metabolic conditions.<sup>27</sup> The care team typically includes metabolic specialists (eg, geneticist or endocrinologist), dietitian, pharmacist, primary care clinician, and psychosocial support clinicians (eg, social work and psychologist). Emergency medicine and critical care medicine clinicians are often involved as well. Given the large interprofessional, multidisciplinary care team, care coordination over the lifespan of the patient is especially important. Thus, understanding roles and responsibilities is essential for effective interprofessional teamwork and collaboration.<sup>2,5,27,28</sup> (Table)

**Table. Roles of the interprofessional, multidisciplinary care team members**

Profession/Specialty	Role
Geneticist/Metabolic physician	<ul style="list-style-type: none"> <li>Leads diagnosis and long-term management</li> <li>Coordinates emergency protocols and metabolic crisis prevention</li> <li>Oversees use of specialized therapies</li> </ul>
Registered dietitian	<ul style="list-style-type: none"> <li>Designs individualized dietary plans to avoid fasting and ensure adequate energy intake</li> <li>Manages MCT supplementation and low-fat/high-carbohydrate regimens</li> <li>Educates family on sick-day management and emergency feeding protocols</li> </ul>
Pharmacist	<ul style="list-style-type: none"> <li>Ensures access to medications</li> <li>Monitors for medication side effects/tolerability and medication-nutrient interactions</li> <li>Supports specialty pharmacy coordination and insurance navigation</li> </ul>
Primary care	<ul style="list-style-type: none"> <li>Monitors growth, development, and routine health needs</li> <li>Coordinates vaccinations and screens for comorbidities</li> </ul>
Social worker	<ul style="list-style-type: none"> <li>Ensures team access to care plans and emergency protocols</li> <li>Assists with insurance navigation</li> <li>Provides connections to support networks</li> <li>Supports transition from pediatric to adult care</li> </ul>
Psychologist	<ul style="list-style-type: none"> <li>Addresses patient and caregiver burden, mental health, and quality of life</li> </ul>
Emergency medicine/Critical care	<ul style="list-style-type: none"> <li>Provides acute management of metabolic decompensation using point-of-care protocols for hypoglycemia, rhabdomyolysis, or cardiac symptoms</li> </ul>
Genetic counselor	<ul style="list-style-type: none"> <li>Provides family planning support and carrier testing for parents</li> </ul>
School nurse	<ul style="list-style-type: none"> <li>Manages feeding and activity at school</li> <li>Monitors for acute symptoms; implements emergency protocol when needed</li> <li>Provides education and support</li> </ul>



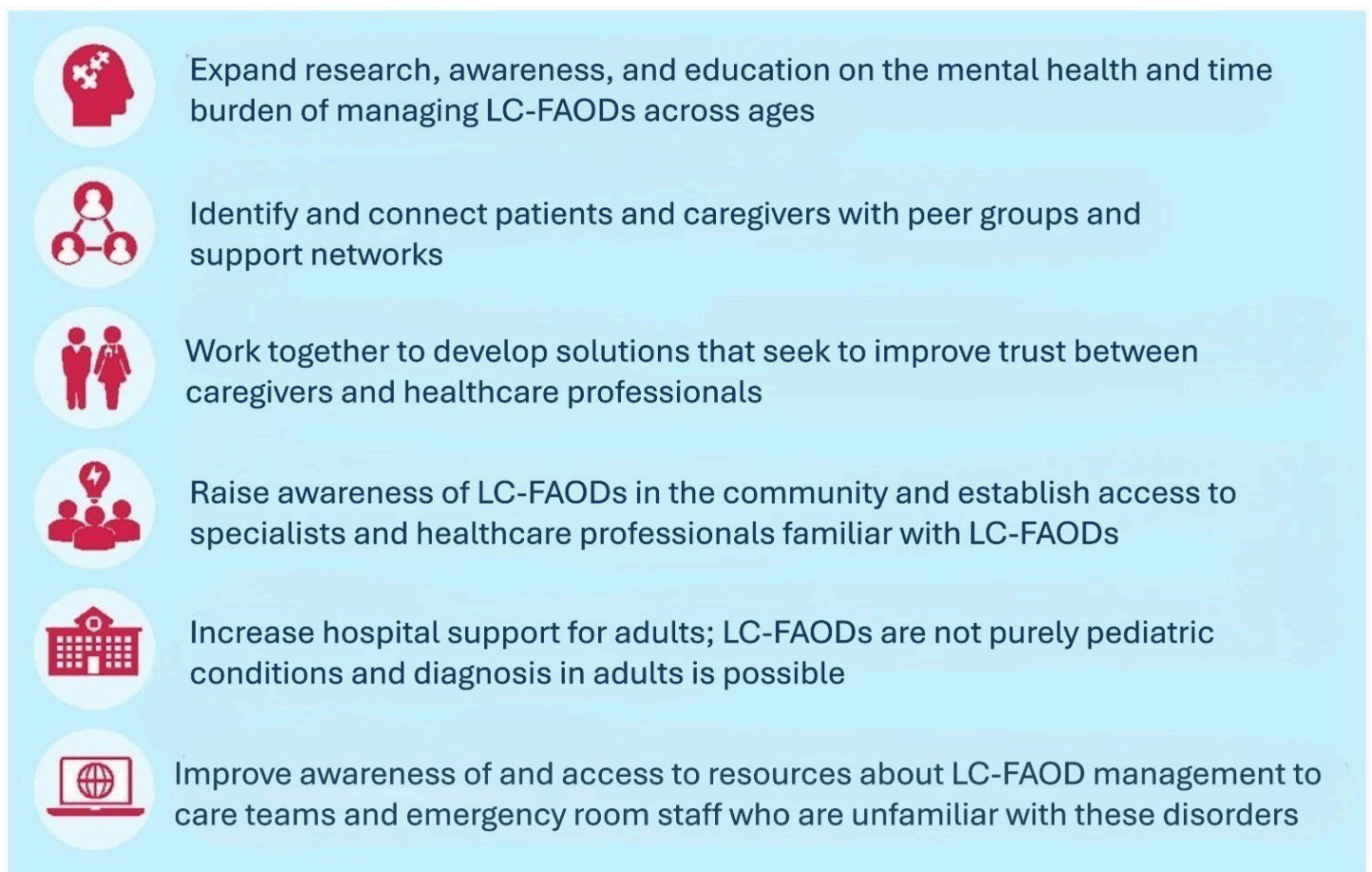
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Clear communication among the care team and with the patient is vital as well. Making the individualized care plan, including dietary plan and emergency and point-of-care protocols, available electronically is key for acute and ongoing patient management. [Note a downloadable point-of-care resource is available with this activity.] Telehealth visits can facilitate patient access to specialty care, thus reducing the travel burden. Moreover, telehealth visits may

obviate families relocating to be close to specialty care. Ongoing education of all team members, including those less frequently involved such as emergency medicine and critical care clinicians, helps ensure that patients are managed appropriately, which may reduce acute care visits and hospitalization, as well as patient and caregiver anxiety. Additional recommendations for improving the care of individuals with LC-FAODs are shown in the **Figure**.<sup>27</sup>

**Figure.** Recommendations for improving the care of individuals with long-chain fatty acid oxidation disorders.



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