ADVANCES IN™

Fatty Acid Oxidation Disorders:

Improving Energy Production Through Alternative Sources

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Advances in™ Fatty Acid Oxidation Disorders: Improving Energy Production Through Alternative Sources was an online activity featuring text and video that was designed to bring the rare genetic disease, long chain fatty acid oxidation disorders (LC-FAOD), into focus for clinicians. With potential treatment options in the pipeline, it is vital that clinicians keep this disease on their radar and are aware of options to manage patients.

EDUCATIONAL OBJECTIVES

1. Define the pathophysiology and symptoms of LC-FAOD
2. Identify disease management options to achieve individualized care for patients with LC-FAOD
3. Apply knowledge of emerging therapies' mechanism of action and adverse drug reactions to clinical management of patients with LC-FAOD

METHODS

- Enduring Advances in™ activity for one year from December 2018 to December 2019
- Moore's Levels 1-4 Outcomes measured Participation, Satisfaction, Declarative and Procedural Knowledge, and Competence

RESULTS

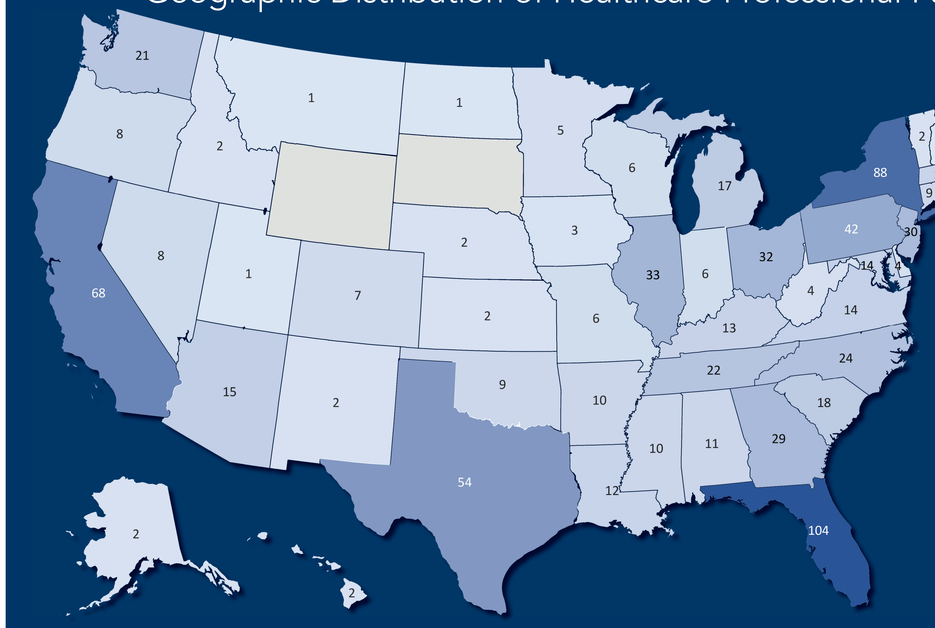
- Based on the posttest results, learners increased their knowledge and competence regarding the mechanism of LC-FAOD, symptoms, and emerging treatment options after completing this activity.
- As this program was focused on pediatrics, the increase in knowledge and competence across pediatricians alone was extremely prominent.

FUTURE EDUCATION

A unique aspect of this particular program was segmenting the learners by location. This allows for a better inclination of where there may be higher populations of patients with LC-FAOD and will allow us to implement geo-targeted education in the future.

Educational Reach

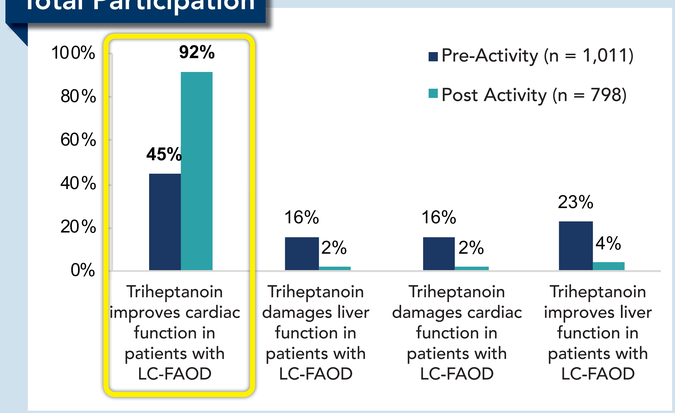
Geographic Distribution of Healthcare Professional Participants



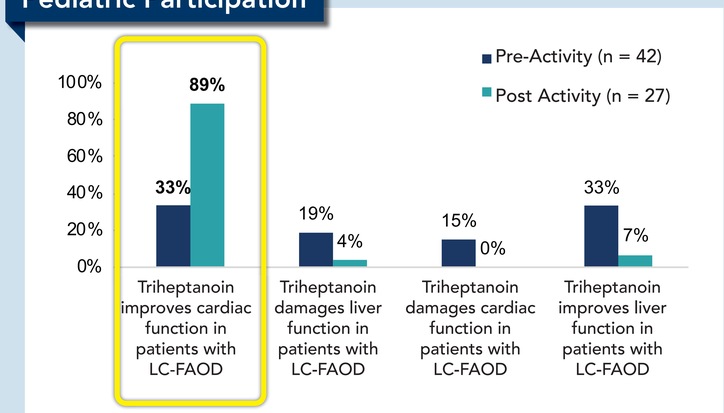
Participants by State			
Alabama	11	Montana	1
Alaska	2	Nebraska	2
Arizona	15	Nevada	8
Arkansas	10	New Hampshire	1
California	68	New Jersey	30
Colorado	7	New Mexico	2
Connecticut	9	New York	88
Delaware	4	North Carolina	24
Florida	104	North Dakota	1
Georgia	29	Ohio	32
Hawaii	2	Oklahoma	9
Idaho	2	Oregon	8
Illinois	33	Pennsylvania	42
Indiana	6	Rhode Island	1
Iowa	3	South Carolina	18
Kansas	2	Tennessee	22
Kentucky	13	Texas	54
Louisiana	12	Utah	1
Maine	2	Vermont	2
Maryland	14	Virginia	14
Massachusetts	13	Washington	21
Michigan	17	West Virginia	4
Minnesota	5	Wisconsin	6
Mississippi	10	Not Specified	87
Missouri	6	Ex-US	223

Advances in™ Rare Diseases: Fatty Acid Oxidation Disorders – Improving Energy Production Through Alternative Sources PER® Final Outcomes Report

Total Participation



Pediatric Participation



Summary of Outcomes Result: Learners demonstrated an increase in knowledge related to phase II clinical trials of triheptanoin supplementation (104% increase across all specialties, 170% increase across pediatric specialty)

Clinical Impact: According to a Phase II Study of Triheptanoin for Treatment of Long-Chain Fatty Acid Oxidation Disorders, C7 improved LV ejection fraction and reduced LV mass at rest, as well as lowering heart rate during exercise among patients with LC-FAODs.

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