Spotlight on Science: Testing a New Form of Medium Chain Fat on Patients with Long Chain Fatty Acid Oxidation Disorders; PI: Gerard Berry MD

Long chain fatty acid oxidation disorders (LC-FAOD) are a group of autosomal recessive diseases in which the body is unable to break down long chain fats into usable energy. These disorders occur in approximately 1 in 14,000 births and can have serious metabolic side effects including rhabdomyolysis, hypoglycemia, hypotonia/weakness and cardiomyopathy. The disorders are now identified through newborn screening, but previously, when identified clinically had a high mortality rate.

Patients with LC-FOAD follow a very low fat diet. They are allowed about 10% of their calories from fat compared to the average American diet of approximately 35% of total calories from fat. The diet is then supplemented with medium chain triglyceride (MCT) oil to ensure they are receiving adequate calories to promote continued growth without the harm of ingesting excessive long chain fats.

A new protocol began in July in the Clinical Translational Study Unit (CTSU, Harvard Catalyst CRC) sponsored by Ultragenyx Pharmaceutical Inc. testing a new form of medium chain fat on patients with LC-FAOD to assess the effect on energy metabolism, tolerance and safety. The first study visit had multiple components that needed to occur in a timely sequence in order to ensure patient safety including the preparation of a nutrient/calorie controlled meal to be served immediately after blood sampling. Prior to the scheduled visit the outpatient Metabolic Dietitian team and the CTSU research nutrition team collaborated to design a meal following the protocol parameters. The meal was then prepared in the metabolic kitchen and served to the patient where he reported that it “tasted just like his Mom cooked it.”

For every first clinical trial visit there can be a few “bumps in the road” while getting underway, however the initial visit of this protocol occurred seamlessly due to the exceptional communication between the study Coordinators, Metabolic Dietitians, CTSU Nursing, Research Pharmacy and the CTSU nutrition team. This collaborative effort is what we strive for each day in the Clinical Translational Study Unit of the Clinical Research Center (CRC).
**CTSU Update: Scheduler Version 2.9 Released**

“Scheduler” is a web-based tool that allows investigators, study staff, and CRC staff to schedule research subjects for CTSU study visits and manage CRC resources. We have received positive feedback from study teams who appreciate the increased access and transparency to the CTSU schedule. Version 2.9 has exciting new features, such as:

- Study staff can cancel visits within 24 hours.
- Study staff have access to the Resource Gantt Charts in the Appointments Module. This feature allows users to see resources already booked.

**CTSU Focus Group: We Want Your Input!**

We held our first focus group regarding the new Scheduling System and it was wonderful to hear the positive things study teams had to say, as well as, what can still be improved. **We still want to hear from you!** We have two more sessions scheduled for August (dates below). The sessions will be located in the CTSU and pizza will be served. Please note that the sessions are limited to 8 people each. Please RSVP to Tina Kim indicating the date you would like to attend.

- Wednesday, August 20th @ 1-2pm
- Thursday, August 21st @ 1-2pm

**Project Management/Study Coordinator Services**

**CATALYST-SUBSIDIZED SERVICES—Click [HERE](#) to Apply**

Subsidized hours for Junior Investigators (Assist. Prof. and below) are now available; guidelines below:

**Project Management Services**
(a mix of RN project manager, non-RN project manager, data manager and study coordinator)
- Junior Investigators eligible, used only once; 150 hrs, up to 1 year
- Senior investigators (Assoc and Prof) can petition for the subsidized hours one time only.
- If Project Management hours are allocated, then the number of subsidized coordinator hours decrease to 100 hours

**Study Coordinator Hours Only**
- Junior Investigators eligible, used only once; 200 hours up to 1 year

Note: Data Management Only requests will always be funded by investigator. If paired with a study coordinator or project manager, then the investigator is eligible; we do not support data entry only requests with study coordinator support.
Dear Colleagues,

As we approach the dog days of summer, our Center remains as busy as ever. Our Introduction to Clinical Research course starts September 9th and this 2 ½ day course provides an introduction to the methods and practice of clinical research and a guide to the relevant BCH resources available to do clinical research at the hospital; a to-do for any clinical researcher at the hospital! We will also have our first annual CRC Poster Day on Day 1 of the course showcasing the great work our staff do with all of you!

Enjoy the rest of the summer,
Ellis and Voula

New Staff

Lindsay Toto, Clinical Research Specialist I
Tess Buccigrosso, Clinical Research Coordinator I

Upcoming Courses

Sept 16, Oct 21, Nov 18, Dec 16
CRC Grand Rounds

Aug 18&21, Sept 29&30, Dec 11&12
Orientation for New Study Coordinators

Aug 20, Sept 24, Oct 15, Nov 11, Dec 10
Coordinator Rounds

Sept 4, Dec 2
I2b2 Information Session

Aug 13, Sept 9, Oct 9, Nov 6, Dec 4
REDCap Information Session

Recent CRC Staff Publications (with CRC staff as first or last author)


For a complete list of CRC staff publications from the past 6 months, please see pages 4 & 5

The Clinical Research Center
Contact CRC@childrens.harvard.edu
Past CRC Newsletters can be found HERE
Recent CRC Staff Publications


