

The Clinical Research Center

Spotlight on Science ~ CTSU New and Exciting Upcoming Studies

"A Multinational, Open-Label, Non-Controlled Trial on Safety, Efficacy and Pharmacokinetics of NNC 0129-0000-1008 in Previously Treated Pediatric Patients with Severe Hemophilia A" PI: Dr. Kapil Saxena

Dr. Kapil Saxena's trial, sponsored by **Novo Nordisk**, is testing a new **Factor VIII drug called N8-GP**. This is an open-label trial that will **enroll 60 patients worldwide** under the age of 12 diagnosed with **severe Hemophilia A** with no signs of inhibitor development. All subjects in this trial will participate during the 26 week "Main Phase" and have the opportunity to continue through the "Extension Phase" which will last until N8-GP becomes commercially available in the United States. **Subjects will receive N8-GP at a fixed dose for prophylaxis treatment.** The first two doses will be administered in clinic then home dosing can be initiated. There are no patients currently enrolled, but the plan is to **enroll 4 patients**.

"N-carbamylglutamate in the Treatment of Hyperammonemia: Neurodevelopment, and Safety in Propionic Acidemia and Methylmalonic Acidemia" PI: Dr. Gerard Berry

Dr. Gerard Berry's trial, sponsored by the **NIH**, is testing a **medication callec Carbaglu**. This is a placebo-controlled trial that will **enroll 30 patients at different medical centers** around the country, **ages 29 days or less**, with **high ammonia levels due to methylmalonic acidemia or prioponic acidemia**. Children will receive Carbaglu or placebo every time they are hospitalized for a high blood ammonia level until 30 months of age. In addition to hospitalizations, children will participate in neurodevelopmental assessments at 9 months, 15 months, 21 months and 30 months of age. Carbaglu is not yet approved by the FDA for use in prioponic acidemia or methylmalonic acidemia, but has been approved by the FDA for the treatment of NAGS deficiency. Information from this research study will help determine whether this drug should be approved by the FDA for use in prioponic acidemia and methylmalonic acidemia. Currently, there are no patients enrolled, but the plan is to enroll 12 patients.

"MAESTRO" PI: Dr. Alexander Opotowsky

Dr. Alexander Opotowsky's "MAESTRO" trial, sponsored by Actelion Pharmaceuticals Ltd. is testing a drug called Macitentan. This is a multicenter, double-blind, randomized, placebo-controlled, parallel-group, Phase 3 study that will enroll 330 subjects in 80 centers in as many as 30 countries. The goal is to evaluate the effects of MAcitentan in Eisenmenger Syndrome To RestOre exercise capacity (MAESTRO). Macitentan has been previously evaluated in subjects with symptomatic pulmonary arterial hypertension, where it has proven effective. This medication is now being tested in subjects who suffer from Eisenmenger Syndrome, i.e., pulmonary arterial hypertension associated with a congenital heart disease. The goal of the study is to determine if it will improve subjects' ability to exercise and if it is a safe treatment for Eisenmenger Syndrome.

Harvard Catalyst Project Management Services ~

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)

- 1. Junior Investigators eligible, used only once; 150 hrs, up to 1 year
- 2. Senior investigators (Assoc and Prof) can petition for the subsidized hours one time only.
- 3. If Project Management hours are allocated, then the number of subsidized coordinator hours decrease to 100 hour

Study Coordinator Hours Only

1. Junior Investigators eligible, used only once; 200 hours up to 1 year

APPLY HERE: catalyst.harvard.edu/services/hccrcrequest/

*When a request is made, our triage team (A. Dauber, C. Williams, A. Simmons) will meet with the investigator to determine the appropriate staffing.

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

Top 10 Reasons you might be a Clinical Researcher...

- 10. You don't organize your sock drawer by color, you stratify by demographic.
- 9. The FDA regulates food too? Who knew!
- 8. You commonly write sentences with more acronyms than real words.
- 7. You have heard the term, "enrollment compete" but never actually experienced it.
- 6. "Modeling" is subject you took in school, and "Subjects" are people who may or may not be models.
- 5. Your match.com profile list inclusion/exclusion criteria rather than like/dislikes.
- 4. Your office is nowhere near anything "clinical"
- 3. You cross-out, date, and initial changes to crossword puzzles rather than erase.
- 2. You envy your bench colleagues because mice show up for their follow-up visits.
- 1. This is the most humorist thing you've read all day!

A picture of our Development & Operations Group reminding us to always have fun on the job and not take things too seriously!

New Data Management Tools for Investigators (click on the name)

Case Report Form Checklist:

- → Intended as a guide for investigators to review their forms with reminders of key elements and to avoid popular mistakes.
- → Complete with a link to a more complete Guidelines for Developing Case Report Forms.

REDCap Database Checklist:

- → Helpful tips to guide the REDCap database development process.
- → Focuses on the standards of programming rather than the basic technologies of REDCap.
- → Recommends database layout, variable construction, naming and coding.

Upcoming Courses...

... Save the Date!!

May 2013
Economic Evaluation

March 19, April 16, May 21 CRC Grand Rounds

September 10, 12, & 13: Introduction to Clinical Research

April 19, May 17, June 14
Orientation for New Study coordinators

September 24 - November 14: Introduction to Biostatistics with SPSS March 20, April 3, May 15 Coordinator Rounds

www.childrenshospital/org./crceducation

New Staff

Tanya Logvinenko, *Biostatistician* Vishnudas Sarda, *Statistical Programmer* Georgios Sideridis, *Survey Methodologist II* Erica Denhoff, *Clinical Research Specialist*Lauren Barrett, *Administrative Associate III*Erin Deneen, *Administrative Associate III*

RAFFLE ~ Enter to win a REDCap Database Build*

Name this location in Boston and you could get a free REDCap Database built!

Email crc@childrens.harvard.edu by Friday,

February 22nd with your answer (the winner will be notified shortly thereafter, good for up to 1 year).

^{*}staff will test and deploy a REDCap databases up to approximately 100 variables; PI provides case reports form or other data collection forms



Dear Colleagues,

As we enter 2013, the CRC has a few things on their docket! We're happy to promote the Project Management Services our team offers and subsidized via the Harvard Catalyst, our FY12 Annual Report is posted here and don't forget about the one-hour hospital-supported consults our team offers. This month we wanted to hi-lite a few studies that are housed on the CTSU, located on Pavilion 6. It is the one spot at the hospital designated for clinical research and our clinical staff do a great job of offering research and clinical support. Lastly, don't forget to enter our raffle!

~ Ellis and Voula

Chiefs' Corner

