

Advocate Children's Hospital
Pediatric Research Progress Report

Date: October 25, 2012

Title of Study: Utility of Serum Ferritin in the Differential Diagnosis of Hemophagocytic Lymphohistiocytosis (HLH)

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Co-Investigators: William Goodell, MD; Melanie Lamprecht, DO; Stephanie Tolentino, DO; and Denise Angst, PhD, RN; Elisabeth Hooper, MD; Oleksandr Kachanov, MD; Laura McCaffry, RN; and Patti Thornton, RN

Funding Organization / Agency: The Paul Seiwert Foundation

Project Summary: HLH is a rare but potentially fatal disease that most commonly affects infants and young children and is very difficult to diagnose and appears to be under diagnosed. Without early detections, it is a disease that results in tissue damage, organ dysfunction, organ failure and is rapidly fatal.

Physician-investigator, Dr. Michael Tsifansky, Pediatric Critical Care Specialist) at Advocate Children's Hospital – Park Ridge is leading a study to determine the frequency of underlying immune system dysregulation in children admitted to the pediatric intensive care unit with severe infections, or suspected infections, with signs and symptoms of shock and/or organ failure. **The funds from the Paul Seiwert Foundation are being used to test these patients for natural killer (NK) cell function and macrophage activation, as well as testing for serum ferritin and other laboratory studies known to be useful in identifying HLH. These unique tests are not covered through insurance.** Those patients found to have possible HLH will be considered for additional treatment. The goals of this research are to better understand early indicators of possible HLH to guide earlier recognition and treatment, and improve patient outcomes.

Progress Report:

This study was approved by the Advocate Institutional Review Board (IRB) on November 28, 2011 and enrollment of research subjects began in February, 2012. Since this time, we have been actively screening potential subjects in the Pediatric Intensive Care Unit (PICU). To date, we have enrolled 11 subjects into our study. Three (27%) of these subjects have had significantly elevated serum ferritin levels (> 500mg/ml), however, none were further diagnosed with HLH.

We are continuing to actively screen subjects. Our enrollment has been challenged by low census in the PICU over the study period to date; we are hoping that this will pick up over the year ahead and to test up to 100 patients. Our goal is to submit an

abstract/paper/poster of preliminary findings of 50 patients to the Society for Critical Care Medicine (SCCM) in July/August 2013. If the poster is accepted it will be presented at the January 2014 SCCM meeting.