Hemophagocytic Lymphohistiocytosis (HLH) Treatment Study

What is the purpose of this study?

Cincinnati Children's is conducting a research study to evaluate the safety and effectiveness of a treatment regimen combining two previous approaches to treat patients with HLH.

Who will be included in this study?

Children and adolescents, birth to 18 years old, diagnosed with HLH, having active disease and not having extensive prior HLH treatment may be able to participate.

What is involved?

The study lasts for 8 weeks with the possibility of additional follow up if you or your child proceeds to bone marrow transplant. The following is a list of procedures that will take place during the study:

Before treatment begins, you/your child will have tests done to confirm the diagnosis of HLH and assess the disease. This will includes drawing blood and bone marrow, looking for infections, testing the immune system, and radiology such as an MRI of the brain and a CT scan of the chest and abdomen.

The treatment regimen is divided into two phases

- Induction therapy, in which medication is given to control the overactive immune system
- Continuation therapy, in which medication is given to keep the immune system quiet for those patients waiting to go to bone marrow transplantation

You or your child will be followed closely throughout the 8 weeks on study to test for response to treatment with routine tests, such as measuring the size of the liver and spleen, blood tests, and looking for side effects of the medications.

There are additional blood, bone marrow and spinal fluid that may be taken for research purposes, in addition to the routine tests for HLH treatment. You will have to give additional permission for these to be taken.

What are the benefits?

You or your child may or may not receive direct benefit from participating in this study. There may be an improved chance of successfully treating your child's HLH. Information learned in this study may benefit other patients with HLH in the future.

Will I get all the facts about the study?

Parents and adult subjects will be given a consent form that thoroughly explains all of the details of the study. The consent form will cover all of the procedures, risks, benefits, compensation, who to contact with questions or concerns and more. A member of the study staff will review the consent form with you and answer your questions. Study procedures will not begin until the parent/guardian or you, the participating subject, have signed this consent form.

What are the risks?

The study treatment may not help to treat your/your child's disease or may make your condition worse. There may be side effects to treatment. A detailed list of possible side effects will be provided to those interested in knowing more about this study.

What is the pay?

You will receive no payment or reimbursement for participation in this research study.

Who should I contact for more information?

Rebekah Kennedy

Cincinnati Children's Hospital Medical Center

Cancer and Blood Diseases Institute

Blood and Marrow Transplantation and Immune Deficiencies

Phone: 513-636-1313

rebekah.kennedy@cchmc.org