NI-0501: A Study to Investigate the Safety and Efficacy of an Anti-IFNγ mAb in Children Affected by Primary Hemophagocytic Lymphohistiocytosis

PURPOSE

Cincinnati Children's is participating in a research study sponsored by NovImmune S.A. to evaluate the safety, tolerability and preliminary efficacy of a new drug (NI-0501) aimed at controlling disease activity in patients diagnosed with primary hemophagocytic lymphohistiocytosis (HLH).

The new drug will be administered on top of a glucocorticosteroid, which is usually part of the current recommended treatment.

WHO CAN PARTICPATE?

Any child or adolescent, up to and including 18 years at diagnosis of HLH.

CONDITIONS

Primary Hemophagocytic Lymphohistiocytosis (HLH)

WHAT IS INVOLVED?

There is an anticipated 8-week treatment period with NI-0501 followed by an additional 4 week follow-up period after the last dose of NI-0501. However, the protocol also allows for a shorter treatment duration, in case hematopoietic stem cell transplantation (HSCT) can be performed before the end of the treatment period, or a longer treatment duration in case transplantation must be postponed. The following is a list of procedures that will take place during the study:

Screening Period

Before treatment begins, the subject will have tests done to confirm the study eligibility and assess the disease status. This will includes drawing blood and other samples, looking for infections, testing the immune system, spinal tap, test/picture to measure heart rhythm (electrocardiogram, ECG), and radiology tests such as ultrasound and chest x-ray (as well as an MRI, if needed).

Treatment Period

The treatment regimen is divided into two phases:

- Treatment Period 1 is characterized by close monitoring during NI-0501 administrations, 6 infusions given every 3 days. In addition, patients will receive Dexamethasone for the duration of the treatment with the study therapy.

- Treatment Period 2 is characterized by less intense monitoring and includes 14 infusions, or less depending on the patient's needs and conditions (Weeks 3 to 8). The time between NI-0501 infusions can be increased; however this will not impact the safety assessments which will occur at least every 6 days.

All patients will be followed closely throughout the 8 weeks on study to test for response to treatment and to look for side effects of the medications with routine and other tests, such as physical examinations, blood tests, ECG and radiology tests.

Follow Up Period

All patients who have received at least one dose of NI-0501 will be monitored for 4 weeks after the last dose.

WHAT ARE THE BENEFITS?

Study subjects 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. Six patients suffering from primary HLH have been treated so far with NI-0501. Of these, 5 are already scheduled for or have undergone transplant.

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 and answer all questions. Study procedures will not begin until the parent/guardian or the participating subject has signed this consent form.

WHO SHOULD I CONTACT FOR MORE INFORMATION?

Sharon Mitchell
Cincinnati Children's Hospital Medical Center
Cancer and Blood Diseases Institute
Blood and Marrow Transplantation and Immune Deficiencies

Phone: 513-636-4174
Sharon.Mitchell@cchmc.org