

Picibanil sclerotherapy: A Multi-center trial

Research Subject Fact Sheet

What is the purpose of this study?

The purpose of this study is to test the activity of Picibanil sclerotherapy (OK-432) in the treatment of macrocystic lymphangiomas. We would like to find out if treatment with Picibanil is related to the cyst(s) going away or to the cyst(s) getting smaller in size.

Picibanil is an investigational drug. An investigational drug is one that has not yet been approved by the Food and Drug Administration (FDA) for commercial use and is available for research use only. It is unknown whether there is benefit from treatment with Picibanil. This drug has been used extensively in Japan and Europe to treat patients with lymphangiomas and other diseases. Approximately 30,000 individuals have been treated with this drug outside of the United States to date. It is not FDA approved in the United States for general use.

What is being done in this study?

Participation in this study is for children (ages 6 mo. to 18 years old) and will be performed over the course of one year. Several visits to the UW Hospital are required, and photographs will be taken at the beginning of this study and at each visit. We will also use a way of looking at the cysts with a machine called a MRI. In addition, we will request some blood and urine tests and an EKG (a way of looking at your heart beat). Injection of Picibanil will be performed under general anesthesia in an operating room using an XRAY (fluoroscopic-guided needle aspiration) to pinpoint the best area/areas to be injected. Recovery of your child will be done in an appropriate recovery area. Your child will have 4 injection sessions of Picibanil during the year of study, unless the cysts completely go away before all four injections are completed or the doctors feel there is a medical reason to stop. All injections will be spaced 6-8 weeks apart. There are two treatment groups in this study: Immediate Treatment or Delayed Treatment. In the Immediate Treatment Group, your child will be entered into the study, while those in the Delayed Treatment Group will be observed for 6 months prior to Picibanil treatment. If the lymphangioma is considered life-threatening, your child will be entered into the Immediate Treatment Group. If you chose to participate in this study, a particular treatment will be randomly assigned to you, based upon a pre-assigned schedule generated by a computer program. As such, you will not have a choice

regarding your particular treatment group. There is no cost to you for the investigational drug. Every attempt will be made to bill your health insurance for other medical costs, such as the MRI scans, lab tests and costs associated with the operating room. However, you should be sure to obtain prior approval from your insurer prior to your first treatment.

How can you participate?

To be eligible for participation in this study, subjects must be within an age range of 6 months to 18 years and must have macrocystic lymphangioma. Subjects cannot have a penicillin allergy; cannot have a history of liver, blood, or kidney diseases; cannot have a hypersensitivity to iodine or other materials that might be used as a contrast material for the XRAYs; cannot be pregnant or nursing; cannot have abnormal laboratory values or a family history of other medical conditions that we can discuss with you.

For more information on this study, including study participation, please call Kari McConnell, RN, Pediatric Nurse Coordinator, at 608-265-8825.

An article about our multicenter trial, which is based at the University of Iowa (Dr. Richard Smith) can be found at the following link: <http://archotol.ama-assn.org/issues/v128n10/abs/ooa10285.html>.