PBTC-039 – Summary for Patients and Families

Title: PBTC-039, Phase II study of Peginterferon alfa-2b (SYLATRON) for pediatric patients with unresectable or recurrent craniopharyngioma

This is a brief summary of a clinical trial, a type of therapeutic research study. Clinical trials include only patients who choose, or whose parents permit them to take part in the research study. Participation is entirely voluntary.

Who might be eligible to participate in PBTC-039?
Children, who are between 18 months and 25 years old and have a craniopharyngioma, which can’t be completely removed with surgery or continues to grow or has come back (recurred) surgery or radiation therapy. Radiation therapy can be very effective against these tumors, but can cause permanent side effects, including effects on learning and metabolism, particularly for those who are young and/or have very large tumors. For those whose tumors have grown back after radiation therapy, there are few effective treatment options. Children’s medical and neurological status needs to be stable to be eligible for PBTC-039.

Study participants will need a variety of medical tests to assess whether they can participate in PBTC-039. These tests may include a medical history, physical examination, Vision tests, blood tests and scans (MRI) of the brain. Other tests may be required if doctors believe they are necessary. About 52 children throughout the United States will take part in PBTC-039.

Why is PBTC-039 being done?
In this research study, we are testing a drug called SYLATRON on cranipharyngioma. We are now studying to see how well it works in children cranipharyngioma. Our hope is that this drug will be a more effective treatment for childhood cranipharyngioma.

The purpose of this research study is to test any good and bad effects of the study drug called SYLATRON. SYLATRON could shrink your tumor but it may also cause side effects. SYLATRON is not FDA approved to treat brain tumors but researchers hope to learn if the study drug can decrease the size of the tumor and cause any symptoms to improve.

What is involved in this study?
All study participants will get the study drug SYLATRON, given as an injection under the skin once a week. A course is 6 weeks long and you will receive 6 injections per course. Every six-week period of treatment is considered one “course” of therapy. You will be taught to administer the injections under the skin so that the medication can be administered at home. You will be required to keep a diary to record when you take SYLATRON.

What are the risks of participating in PBTC-039?
Doctors watch study participants very carefully for any side effects or other problems. However, doctors do not know all the side effect that may occur. Side effects may be mild or very serious. Other medications may be given to lessen side effects. In some cases, side effects may be long lasting or may never go away. This is also a risk of death. Many side effects go away soon after your child stops taking SYLATRON.
Almost all participants experience flu-like symptoms, especially during the first week or two of receiving the SYLATRON. These can include fever, chills and shivering, headache, vomiting, abdominal pain, muscle aches or tiredness. At the dose that you will receive, these side effects are usually mild. The symptoms can start 1–2 hours after receiving the injection, and generally peak 4–8 hours after the injection. These symptoms do not usually last longer than 18 hours. Symptoms tend to lessen over time. You will be given instructions on medicines to use to lessen these side effects. You will be given acetaminophen prior to the SYLATRON dose, as needed, to lessen fever, chills, headache and achiness.

**QUESTIONS ABOUT PBTC-039?**
If you would like more information, please contact the PBTC member institution closest to you. You can also contact the doctor in charge of this study:

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Other information is available through the following:

The National Cancer Institute’s (NCI) Cancer Information Service at 1-800-4-CANCER (1-800-422-6237) or through the NCI’s Web site such as www.cancer.gov and www.cancer.gov/clinicaltrials. There is additional accurate and reliable information at www.cancernet.org