Summary for Patients and Families

PBTC-060: A Pilot Study of Safety, Tolerability, and Immunological Effects of SurVaxM in Pediatric Patients with Progressive or Relapsed Medulloblastoma, High Grade Glioma, Ependymoma, and Newly Diagnosed Diffuse Intrinsic Pontine Glioma

The Pediatric Brain Tumor Consortium (PBTC) is conducting this research study. The PBTC is a group dedicated to improving the treatment of childhood brain tumors. It comprises fifteen academic centers and children's hospitals in the United States and Canada.

The following is a summary of a clinical trial, a therapeutic research study. Clinical trials include only patients who choose or whose parents permit them to participate in the research study. Participation is entirely voluntary.

Who might be eligible to participate in PBTC-060?

Patients with a brain tumor called medulloblastoma, high-grade glioma, or ependymoma that has worsened or returned after other treatments are eligible to participate in this study. These patients must have tumor tissue that tests positive for a protein called survivin during screening in order to be eligible. Survivin can be found on up to 95% of glioblastomas and other types of cancer but is not found in normal cells. These patients must also have received prior chemotherapy, immunotherapy, radiotherapy, or other treatment modalities.

Patients with newly diagnosed diffuse intrinsic pontine glioma (DIPG) are also eligible to participate in this study. DIPG patients may not have tumor available and will not be tested for survivin at screening. Patients with newly diagnosed DIPG must have completed radiation therapy.

Patients must be 1- 21 years of age at the time of screening, which involves laboratory tests on tumor tissue. Patients will undergo clinical evaluations to confirm adequate organ and bone marrow function prior to starting the trial.

Why is PBTC-060 being undertaken?

There is no effective standard treatment for medulloblastoma, high-grade glioma, ependymoma, or newly diagnosed DIPG. This research will examine the safety of a vaccine called SurVaxM. This vaccine treatment will be combined with Montanide ISA 51 and sargramostim to see if these medications are safe and tolerated without severe side effects in children.

What is involved in this study?

Participating patients will receive subcutaneous injections of SurVaxM (SVN53-67/M57-KLH) combined with Montanide ISA 51. The SurVaxM/Montanide injection will be followed immediately by a subcutaneous sargramostim injection near the vaccine injection site.

Patients will get a subcutaneous SurVaxM/Montanide injection at the start of the study and every 2 weeks for 6 weeks (for a total of 4 doses) followed by 2 weeks of follow up. At the same patients will also get a second subcutaneous injection of a medicine called sargramostim (known as GM-CSF) near the vaccine injection site. This is called the Priming Phase.

If a patient completes the Priming Phase without severe side effects and the disease stays the same or improves, patients can continue to the Maintenance Phase. During the Maintenance Phase, patients will get a SurVaxM/Montanide ISA 51 dose along with a GM-CSF dose about every 8 weeks for up to two years.

All patients will be monitored for acute allergic reactions immediately following vaccination. Outpatients will be observed for at least 60 minutes after each vaccine dose. Blood pressure and heart rate will be checked after the first dose of SurVaxM emulsion with Montanide ISA 51 plus sargramostim. Patients will complete an injection site reaction diary for 4 days following each injection.

What are the risks of participating in PBTC-060?

There is a risk that you could have side effects from SurVaxM/Montanide ISA 51. These side effects may be worse and may be different than you would get with other treatments for your cancer. Some of the most common side effects that the study doctors know about are:

- Tiredness
- Fever
- Swelling and redness at the site of the medication injection
- Itching
- Thickening of the skin at the site of the medication injection
- Hives
- Headache
- Back pain
- Pain in joints and muscles.
- Allergic reactions

The study doctors do not know who will or will not have side effects. Some side effects may go away quickly, some may last a long time, and some may never go away. Some side effects may be mild. Other side effects may be severe and even result in death. You can ask your study doctor questions about side effects at any time. If you notice or feel anything different, tell your study doctor. They can check to see if it is a side effect. Your study doctor will work with you to treat your side effects. Your study doctor may adjust the study drugs to reduce side effects.

QUESTIONS ABOUT THIS STUDY?

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 National Cancer Institute's Cancer Information Service at 1-800-422-6237 or TTY: 1-800-332-8615 or through the National Cancer Institute's websites www.Cancer.gov and www.cancer.gov/clinicaltrials.