

PBTC-032 - A SUMMARY FOR PATIENTS AND FAMILIES

Title: PBTC-032 Phase II Clinical Trial Evaluating the Efficacy and Safety of GDC-0449 in Children with Recurrent or Refractory Medulloblastoma

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-032?

Children may be eligible who are between 3 and 21 years of age, who have been diagnosed with a brain tumor called medulloblastoma, which continues to grow or has come back (recurred) after being treated with standard therapies (radiation and chemotherapy), and for which there is no curative therapy. Eligible patients must be finished with therapies at least 4 weeks prior and must be able to swallow capsules. Patients will also need to have brain tumor tissue available from a prior surgery for laboratory research. Children's medical and neurological status needs to be stable to be eligible for PBTC-032.

Study participants will need a variety of medical tests to assess whether they can participate in PBTC-032. These tests may include a medical history, physical examination, blood and urine tests and scans (MRI) of the brain and spine. Other tests may be required if doctors believe they are necessary. About 50 children throughout the United States will take part in PBTC-032.

WHY IS PBTC-032 BEING DONE?

Currently, there are few effective treatments for medulloblastoma that recurs or is resistant to standard therapies. PBTC-032 tests a new drug called GDC-0449 to see how well it works in children with medulloblastoma. GDC-0449 has not been studied extensively in children, and the hope is that this drug will be a more effective treatment for this type of childhood brain tumor.

Specifically, the goals of PBTC-032 are to

- See what effects (good or bad) GDC-0449 has on your child and your child's brain tumor;
- Learn how the body processes GDC-0449 by studying fluids of the body and brain tumor tissue in the laboratory;
- Measure levels of certain cells and proteins in the blood to see if changes are related to how the brain tumor responds to GDC-0449.

WHAT IS INVOLVED IN THIS STUDY?

GDC-0449 comes in a hard capsule which must be swallowed whole once a day. Your child will take GDC-0449 every day for 28 days. Doctors will give you a diary to keep a record of all of the doses taken by your child. The 28-day period when your child takes GDC-0449 will be repeated every 4 weeks. Each 4-week period is considered one "course" of therapy. Patients will receive up to 26 courses of therapy (104 weeks).

Prior to the study, in addition to the medical tests that are of routine cancer care, your child will receive a scan (MRI) of the right knee and a routine dental evaluation to provide a baseline measurement to assess the growth of bone and teeth while he/she is in the study. These tests are not part of routine cancer care and will be done because your child is participating in PBTC-032. During the clinical trial, your child will receive both the routine medical tests and additional tests to understand how your child is responding to GDC-0449.

WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-032?

Doctors watch study participants very carefully for any side effects or other problems. However, doctors do not know all the side effects which may occur. Side effects may be mild or very serious. In some cases, side effects may be long lasting or may never go away. There also is a risk of death. Many side effects may go away soon after a child stops taking the GDC-0449. Some of the side effects of GDC-0449 may be problems in the growth of bone and teeth and problems with muscles, such as spasms. Other side effects of GDC-0449 may be diarrhea, fatigue, nausea, vomiting and swelling and pain in the arms and legs. The health care team may give study participants medicines to help lessen side effects. Doctors will notify parents and patients immediately of any important information or treatment findings discovered during the study that may affect their willingness to continue to participate.

QUESTIONS ABOUT PBTC-032?

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. There is additional accurate and reliable cancer information at www.cancernet.org.