

PBTC-025B - A SUMMARY FOR PATIENTS AND FAMILIES

Title: PBTC-025B-A Phase II Clinical Trial Evaluating the Efficacy and Safety of GDC-0449 in Adults 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 to take part in the research study. Participation is entirely voluntary.

WHO MIGHT BE ELIGIBLE TO PARTICIPATE IN PBTC-025B?

Adults may be eligible who are 22 years of age or older and who have a diagnosis of a brain tumor called a medulloblastoma that is recurrent, progressive or not responding to standard treatment and for which there is no known curative therapy. To be eligible, patients will also need to have brain tumor tissue available from a prior surgery for laboratory research. Patients may be eligible who have finished chemotherapy or immunotherapy 4 weeks prior to enrolling in PBTC-025B. Patients must have finished craniospinal radiation therapy 3 weeks prior, 8 weeks prior for local radiation therapy and 2 weeks prior for focal radiation.

To participate in PBTC-025B, other tests and information will be required, including a medical history, physical examination, blood tests and scans (MRI) of the brain and spine, spinal fluid exam (CSF), and a pregnancy test for females. Additional tests may be required if doctors believe they are necessary. About 50 adults throughout the United States will take part in PBTC-025B.

WHY IS PBTC-025B BEING DONE?

The purpose of this study is to test a new drug called GDC-0449 to determine whether it is safe and effective in adult patients with recurrent or refractory (resistant to various standard treatments) medulloblastoma. Studies show that GDC-0449 blocks a cell signaling pathway that has prevented or slowed the growth of several types of human cancer cells grown in animals. GDC-0449 has been previously studied in adults. Our hope is that GDC-0449 is a more effective treatment for this type of brain tumor.

Specifically, PBTC-025B will

- Determine what effects (good and bad) GDC-0449 has on you and your tumor.
- Determine how the body processes GDC-0449 by studying fluids of the body and the brain tumor tissue.
- Measure levels of certain cells and proteins in the blood to determine whether changes are related to brain tumor response to GDC-0449.

WHAT IS INVOLVED IN THIS STUDY?

PBTC-025B adult participants will receive GDC-0449 in a hard gelatin capsule to be swallowed once a day for 28 days. Doctors will give you a diary to keep a record of all of the doses you take. The 28-day period when you take 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 (2 years or 104 weeks).

Throughout the study courses, participants will have routine blood tests, history and physical exams, and pregnancy tests for females. MRIs of the brain and spine and will also be carried out as will spinal fluid examination, if safe to perform. Follow-up treatment will consist of a routine physical exam and history bimonthly for up to 12 months following the last dose of GDC-0449.

WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-025B?

Doctors watch study participants very carefully for any side effects or other problems. However, doctors do not know all the side effects 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. There is also a risk of death. Many side effects may go away soon after you stop taking GDC-0449.

Some of the common side effects of GDC-0449 are muscle spasms/twitching, taste changes, hair loss, runny nose and heart arrhythmia. You should not become pregnant or father a baby while on this study because how GDC-0449 may affect an unborn baby is unknown. You should talk to your study doctor about any side effects that you have while taking part in the study.

QUESTIONS ABOUT PBTC-025B?

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 information about cancer at www.cancernet.org.