Title: PBTC-029 A Phase 1 Study of AZD6244 in Pediatric Patients with Recurrent or Refractory Low Grade Gliomas

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-029?

Children may be eligible who are between 12 and 21 years of age and who have been diagnosed with a low grade glioma or an optic pathway glioma, which continues to grow or has come back (recurred) after receiving radiation therapy or other standard treatment. Eligible patients must be able to swallow capsules and have neurological deficits that are stable for at least 1 week prior to study registration.

Patients will need medical tests to assess whether they can participate in PBTC-029. These tests may include a medical history, physical examination, ophthalmology examination, pulse oximetry, blood and urine tests and scans (MRI) of the brain and spine. Other tests may be required if doctors believe they are necessary. About 15-25 children throughout the United States will take part in PBTC-29.

WHY IS PBTC-029 BEING DONE?

Currently, there are few effective treatments for this type of brain tumor. This study tests a new drug called AZD6244 to see how well it works in children with brain tumors. AZD6244 has not been studied in children before, and the hope is that this drug will be a more effective treatment for childhood brain tumors.

Specifically, the goals of PBTC-029 are to

- Find the highest dose of AZD6244 that can be given without causing severe side effects;
- Learn what side effects (good or bad) may occur when patients take AZD6244;
- Learn how the body processes AZD6244 and how certain tumors respond to AZD6244.

WHAT IS INVOLVED IN THIS STUDY?

AZD6244 comes in capsule form and must be swallowed whole on an empty stomach twice per day for 28 consecutive days. Your child will receive up to 13 months of therapy.

The doctors will give you a diary to record the time the drug was taken and the dose to help you keep accurate records.

During the study, your child will have routine medical tests including a physical exam, blood tests, scans of the brain and spine, test of the spinal fluid, and a pregnancy test for females of childbearing potential. These tests and procedures are all routine cancer care, but are being done more often to monitor your child during this study. Doctors may also request special tests to help your child or to help them understand how the drug is working.
WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-029?

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 go away soon after your child stops taking the AZD6244.

Some of the side effects of AZD6244 are acne, diarrhea, fatigue, nausea, vomiting and swelling of the arms and legs. Your child should not become pregnant or father a baby while on this study because how AZD6244 may affect an unborn baby is unknown. You should talk to your child’s study doctor about any side effects that your child has while taking part in the study.

QUESTIONS ABOUT PBTC-029?

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 at www.cancernet.org.