

PBTC-027 - A Summary for Patients and Families

Title: A Phase I Study of ABT-888, an Oral Inhibitor of Poly(ADP-ribose) Polymerase and Temozolomide in Children with Recurrent/Refractory CNS Tumors

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-027?

Children who have a primary CNS tumor that is recurrent or resistant to standard therapy may be eligible to participate in PBTC-027. Eligible patients must be 21 years of age or younger and may not be pregnant or nursing. For at least one week prior to participating in PBTC-027, patients may not have received any anti-cancer therapy or be taking any experimental agent. To participate, patients must be able to swallow medicine by mouth. Children's medical and neurological conditions must be stable at the time they begin to participate in the trial. Between 24 and 30 children throughout the United States will take part in this study.

Patients will need medical tests to assess whether they can participate in PBTC-027. 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.

WHY IS PBTC-027 BEING DONE?

This study tests an experimental drug called **ABT-888** that has been studied in combination with temozolomide (a type of chemotherapy) in adults. Our hope is that ABT-888 will increase a tumor's sensitivity to temozolomide and that this combination of drugs will be a more effective treatment for childhood brain tumors. This is the first time this combination is being studied in pediatric patients.

Specifically, PBTC-027 will:

1. To determine the maximum doses of ABT-888 and temozolomide combined that can be given to children with gliomas;
2. To understand how children's bodies process ABT-888 through blood and other tests so as to know how best to help children with brain tumors in the future;
3. To learn what side effects may occur when ABT-888 and temozolomide are given together;
4. To understand more about certain childhood brain tumors and how they may respond to this combination of drugs through laboratory studies of brain tumor tissue, if available, from your child's prior surgery.

WHAT IS INVOLVED IN THIS STUDY?

Every day for five days, study participants will take one dose of ABT-888 by mouth in the morning and one dose in the evening. Each day they will also swallow temozolomide capsules 60-90 minutes after the morning dose of ABT-888. This 5-day a week schedule of medication may continue for up to 52 weeks. After that, you and your doctor can determine if your child may need treatment for another 52 weeks.

Study participants will need routine blood and urine tests and scans of the brain and spine at specified times during the study. Doctors will ask parents' permission to use small amounts of blood and brain tumor tissue to study the biology of the tumor and how the treatments affect the body.

WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-027?

Doctors watch study participants very carefully for any side effects or other problems that may occur. However, doctors do not know all the side effects which may come about. 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 ABT-888 and temozolomide.

The drugs given in PBTC-027 most often can result in nausea and vomiting. Weight loss, fatigue and other side effects can occur. Temozolomide can cause low blood cell counts and muscle aches and pains. Known side effects are listed in more detail in the consent form for PBTC-027.

There may be unknown risks and discomforts involved in participating in this or any clinical trial. The health care team may give 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-027?

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:

The National Cancer Institute's Cancer Information Service at 1-800-4-CANCER (1-800-422-6237) or TTY: 1-800-332-8615 or NCI website: cancer.gov

Comprehensive clinical trial information is available at NIH's website clinicaltrials.gov

Accurate cancer information is available from the American Society of Clinical Oncology Cancer.Net