PBTC 047 – SUMMARY FOR PATIENTS AND FAMILIES

Title: Phase 1 Trial of Panobinostat in Children with Diffuse Intrinsic Pontine Glioma

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-047?

Patients may be eligible who are between 2 and 21 years of age, who have been diagnosed with DIPG brain tumors which continue to grow or have come back, after being treated with radiation. Eligible patients must be finished with other therapies, depending on the type of therapy, between 7 days prior and 3 months prior to enrollment on this study. The neurological status needs to be stable for at least 3 days prior to enrollment.

Why is PBTC-047 being done?

Malignant brain tumors in children have a poor prognosis, bad side effects often accompany most chemotherapy or immunotherapy regiments, and overall survival for children with tumors which grow or come back is low. PBTC-047 tests a drug called panobinostat to see how well it works in children with these tumors. The panobinostat drug has not been studied in children. The hope is that this drug may be a more effective treatment for these types of brain tumors in children.

Specifically, the main goals of this research study are to:

- Determine which dose of the panobinostat drug is safe for children
- Learn what side effects (good or bad) may occur when patients take the panobinostat drug
- Learn how the body processes the panobinostat drug by studying blood in the laboratory

What is involved in this study?

The panobinostat drug is taken by mouth, 3 times per week for 3 weeks, followed by 1 week of rest from taking the drug. This 4 week period is considered one “course” of therapy, and it can be repeated for up to 26 courses in a row (about 2 years).

Prior to and during the study, you or your child will have routine medical tests including a physical exam, blood tests, cardiac tests, brain scan (MRI), and a pregnancy test for females of child-bearing potential. Most of these tests and procedures are all part of routine cancer care, but they may be done more often to monitor you or your child during the PBTC-047 study.

What are the risks of participating in PBTC-047?

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 someone stops taking the panobinostat drug.

Some of the side effects of the panobinostat drug may include infections, loss of appetite, insomnia, dizziness, headache, heart palpitations, light-headedness or fainting, cough, shortness of breath, diarrhea, nausea, vomiting, abdominal pain, heartburn, tiredness, swelling of the arms and legs, fever, weakness, weight loss, low platelets, low red blood cells, low white blood cells, septic shock, painful blisters around your face or in your mouth that may cause painful swallowing, candidiasis (a fungal infection), low thyroid, high blood sugar, dehydration, bleeding in the brain which may cause headache or confusion, fainting, muscle twitching, taste changes, bleeding around the eyes that can make them look red, abnormal heart beat, high blood pressure, low blood pressure and feeling faint upon standing, small rattling sounds in the chest, wheezing, bloody nose, stomach or intestine bleeding, inflammation of the lining of the stomach, chapped lips, abdominal distension, dry mouth, flatulence, abnormal liver function, skin lesions/rash or redness of the skin, joint swelling, kidney failure, blood in urine, urinary incontinence, chills, malaise, hemorrhagic shock, gastrointestinal and pulmonary hemorrhage-related death, bleeding into the lung, coughing up blood, inflammation of the large intestine, vomiting blood, and pain from the stomach and/or intestines.

The health care team may give study participants medicines to help lessen side effects. Doctors will notify patients 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-047?

If you would like more information, please contact the PBTC member institution closest to you. You can also contact the doctor in charge of the study:

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Other information will be available through the following:
The National Cancer Institute's (NCI) Cancer Information Service at 1-800-4-CANCER (1-800-422-6237) or through the NCI's Web site such as www.cancer.gov and www.cancer.gov/clinicaltrials. There is additional accurate and reliable information at www.cancernet.org