

## **PBTC 043 – SUMMARY FOR PATIENTS AND FAMILIES**

### ***Title: A Phase I Trial of Pomalidomide for children with recurrent, progressive or 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-043?**

Patients may be eligible who are between 3 and 20 years of age, who have been diagnosed with brain tumors except radiation induced gliomas, which continue to grow or have come back, after being treated with standard therapies, such as chemotherapy and radiation. Eligible patients must be finished with other therapies, depending on the type of therapy, between 7 days prior and 6 months prior to enrollment on this study. The neurological status needs to be stable for at least 1 week prior to registration.

### **Why is PBTC-043 being done?**

Malignant brain tumors in children have a poor prognosis, bad side effects often accompany most chemotherapy or immunotherapy regimens, and overall survival for children with tumors which grow or come back is low. PBTC-043 tests a drug called pomalidomide to see how well it works in children with these tumors. The pomalidomide 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 pomalidomide drug is safe for children
- Learn what side effects (good or bad) may occur when patients take the pomalidomide drug
- Learn how the body processes the pomalidomide drug by studying blood in the laboratory

### **What is involved in this study?**

The pomalidomide drug is taken by mouth, once a day, every day 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, brain scan (MRI), and a pregnancy test for females of child-bearing potential. 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-043 study.

### **What are the risks of participating in PBTC-043?**

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 pomalidomide drug. Some of the side effects of the pomalidomide drug may include tiredness, anemia, constipation, diarrhea, nausea, vomiting, swelling of arms & legs, fever, pain, infections, bruising, bleeding, loss of appetite, muscle spasms, dizziness, cough, shortness of breath, itching, rash, and others. 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-043?**

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:

Jason Fangusaro, MD  
Ann and Robert H. Lurie Hospital of Chicago  
Telephone: 312-227-4846  
E-mail: [jfangusaro@luriechildrens.org](mailto:jfangusaro@luriechildrens.org)

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](http://www.cancer.gov) and [www.cancer.gov/clinicaltrials](http://www.cancer.gov/clinicaltrials). There is additional accurate and reliable information at [www.cancernet.org](http://www.cancernet.org)