**PBTC-018 - A SUMMARY FOR PATIENTS AND FAMILIES**

**TITLE: A Phase 1 Trial of CC-5013(Lenalidomide) in Pediatric Patients with Recurrent or Refractory Primary CNS Tumors**

This summary briefly describes a clinical trial (a type of 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 FOR PARTICIPATION IN PBTC-018?

Children less than 21 years of age may be eligible to participate in PBTC-018. Eligible patients will have malignant brain or spinal cord tumors that were treated previously but have returned or been unresponsive to treatment. Patients who are pregnant or breast-feeding cannot participate in this clinical trial. 40-60 pediatric brain or spinal cord tumor patients from all over the U.S. will take part in PBTC-018.

## WHY IS PBTC-018 BEING DONE?

PBTC-018 tests a new investigational drug called CC-5013 (or lenalidomide). CC-5013 is closely related to another drug called thalidomide. Thalidomide was first developed as a sedative to produce a calming effect. Later on, thalidomide was found to cause birth defects in the children of pregnant women who had taken it during pregnancy.

Since then, researchers have learned that thalidomide can block the formation of new blood vessels which tumor cells need to grow and survive. The exact way that thalidomide fights cancer cells is not known, but is probably a combination of effects on the blood vessels and on the body's immune system. When thalidomide was given to adults with brain tumors, some patients' tumors shrank or stopped growing. This beneficial effect lasted only a few months in most patients.

CC-5013 is chemically related to thalidomide, but, in laboratory tests, is more effective in preventing new blood vessels from growing. When CC-5013 was studied in animals, it showed no evidence of causing birth defects.

CC-5013 is being studied in PBTC-018 in the hopes that will be a useful drug to treat pediatric brain and spinal tumors. PBTC-018 is the first time CC-5013 is being studied in children.

The purposes of this study are to:

- Find the highest dose of CC-5013 that can be given safely without causing severe side effects
- See what effects (good or bad) CC-5013 has on a child and a child's tumor
- Learn how a child's body processes CC-5013 by studying fluids of the body

- Measure levels of certain cells and proteins in the blood to see if changes are related to how the tumor responds to CC-5013.
- See if there are any changes in brain scans (MRI, PET) related to the CC-5013

**WHAT IS INVOLVED IN PBTC-018?**

CC-5013 comes in capsule form and needs to be swallowed whole. The capsules cannot be chewed or opened.

Study participants will receive CC-5013 every day for 21 days (3 weeks), followed by a 7-day (one week) rest period. This four week cycle will be repeated 12 times, taking approximately one year. When the year is over, study participants will be followed closely and need standard tests and procedures to monitor their condition.

**WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-018?**

Doctors monitor patients carefully for any side effects or other problems. They may give patients other medicines to lessen the side effects.

Some of the side effects of CC-5013 may include abnormal blood counts, blood clots, heart changes, breathing changes, rash, stomach upset, fatigue, muscle problems, headache, dizziness, and problems with urination. It is unclear from past studies whether these side effects were caused by CC-5013 alone or in combination with other drugs.

**QUESTIONS ABOUT PBTC-018?**

If you would like more information, please contact the PBTC member institution closest to you. You can also contact the doctors 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:

- cancer.gov
- CancerTrials: comprehensive clinical trial information
- CancerNet: accurate cancer information