PBTC-019 - A SUMMARY FOR PATIENTS AND FAMILIES

TITLE: Phase I Pharmacokinetic Optimal Dosing Study of Intrathecal Topotecan for Children with Neoplastic Meningitis

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 FOR PARTICIPATION IN PBTC-019?

Children between 3 and 21 years of age may be eligible to participate in PBTC-019. Eligible patients have cancer that has spread to the lining of the brain and/or spinal cord (neoplastic meningitis). This condition may be due to a brain tumor, leukemia, lymphoma or any other type of tumor that has spread to the lining of the brain or spinal cord. Up to 49 patients throughout the U.S. will take part in this study.

Medical tests to assess whether children can be enrolled in PBTC-019 may include a medical history, physical examination, blood tests and scans (MRI) of the brain and spine. Other tests may be required if doctors believe they are necessary.

The investigational drug in PBTC-019 is given directly into the spinal fluid. For this reason, eligible patients must have no blockage in the flow of the fluid surrounding the brain and spinal cord (cerebrospinal fluid or CSF). A patient will need to have a test called a CSF flow study, a type of scan, to assess whether there is a blockage.

Patients also cannot have cancer cells in their bone marrow. Children with leukemia or lymphoma will need a bone marrow test to determine whether there are no cancer cells there. If a child has another kind of tumor, a bone marrow test may be performed if doctors find it necessary.

Eligible patients also must have an Ommaya reservoir or similar type of surgically implanted catheter through which to inject medication or to withdraw CSF from the fluid chambers in the head. If patients do not have an Ommaya reservoir or similar catheter, they will have to have one surgically implanted in order to be eligible to participate in PBTC-019.

WHY IS PBTC-019 BEING DONE?

The purpose of PBTC-019 is to test an investigational drug called topotecan on cancer that has spread to the lining of the brain and/or spinal cord. There is no known curative therapy for this condition, which is called neoplastic meningitis. When given by vein (intravenously), topotecan shows anti-tumor activity against a wide variety of cancers in children and adults. In PBTC-019, study participants will receive topotecan directly into the CSF, the fluid surrounding the brain and the spine. This way of giving a drug is called “intrathecal administration” and is an attempt to get the drug to the cancer cells directly.
The purposes of this study are to:

- Find the best dose of topotecan that can be safely given directly into the CSF of children whose cancer has spread to the lining of the brain and/or spinal cord
- Find out what effects (good or bad) topotecan has when given directly into the CSF in children with neoplastic meningitis
- Determine if intrathecal administration of topotecan is beneficial to these patients
- Understand better how a child’s body handles topotecan after intrathecal administration
- Evaluate the CSF for markers of tumor spread.

**WHAT IS INVOLVED IN PBTC-019?**

Children participating in PBTC-019 will receive topotecan directly into the CSF through an Ommaya reservoir or similar type of catheter.

PBTC-019 has 3 phases, called “Induction,” “Consolidation,” and “Maintenance.” The study lasts about 54 weeks or one year.

Induction lasts 4 weeks. During the first and third weeks of Induction, participants will receive topotecan 5 days in a row. They will not receive topotecan during the second and fourth weeks of Induction. Doctors will evaluate participants’ disease at week 4. If it is stable or improves, participants will then move to Consolidation.

Consolidation lasts 6 weeks. During the first and fourth weeks of Consolidation, participants will receive topotecan 5 days in a row. They will not receive topotecan during weeks 2, 3, 5 or 6. Doctors will evaluate participants’ disease at week 6. If it is stable or improves, participants will then move to Maintenance.

Maintenance lasts 44 weeks. Every fourth week, patients will receive topotecan 5 days in a row.

Doctors will request permission from parents and patients to study very small amounts of CSF from participants to find out what effects topotecan is having on the cancer cells and on the body.

**WHAT ARE THE RISKS OF PARTICIPATING PBTC-019?**

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 most frequent side effects of topotecan after intrathecal administration include: nausea and vomiting, headache, back pain, fever, fatigue, inflammation at the injection site or of the covering of the brain and spinal cord, abnormal blood counts and other problems.

**QUESTIONS ABOUT PBTC-019?**

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:
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