

PBTC-026 - A SUMMARY FOR PATIENTS AND FAMILIES

Title: A Feasibility Study of SAHA combined with Isotretinoin and Chemotherapy in Infants with Embryonal Tumors of the Central Nervous System

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-026?

Children may be eligible who are between 2 months and 4 years of age and who have a newly diagnosed high risk medulloblastoma or primitive neuroectodermal tumor (PNET). Eligible patients must not be currently taking or have received any prior treatment for their tumor other than surgery. Patients also must have brain tumor material available from the surgery prior to the trial enrollment so that it might be studied. Patients' medical and neurological condition must be stable at the time they begin to participate in the trial.

Patients will need medical tests to assess whether they can participate in PBTC-026. 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. About 45 children throughout the United States will take part PBTC-026.

WHY IS PBTC-026 BEING DONE?

New drugs called SAHA and isotretinoin are being studied in the treatment of embryonal tumors of the central nervous system, also known as medulloblastoma or PNET. Laboratory studies show that these drugs are effective in treating these tumors and are more effective when used in combination with standard chemotherapy drugs. Animal studies show that medulloblastoma tumor cells are more likely to die when SAHA and isotretinoin are taken together. PBTC-026 is being done to find out if these new drugs and standard chemotherapy together can improve the outlook for very young children with embryonal brain tumors.

Specifically, PBTC-026 will

- Determine how safe and effective the combination of SAHA, isotretinoin and standard chemotherapy drugs are in the treatment of children's medulloblastoma/PNET;
- Analyze tumor tissue to see if there are genetic differences in tumors that can help researchers learn more about high-risk medulloblastoma/PNET and how to treat it.

Doctors and researchers are testing SAHA and isotretinoin in combination with chemotherapy in children in the hopes that it will be a more effective treatment for high risk medulloblastoma/PNET than currently available therapies.

WHAT IS INVOLVED IN THIS STUDY?

Study participants will take SAHA in an oral suspension and isotretinoin in a capsule. Standard chemotherapy drugs are given through a vein. PBTC-026 is designed in three treatment phases, each of which is divided into "courses" of treatment:

1. Induction Phase – 3 courses (21 days each course)

In each course, SAHA and isotretinoin are given first then followed by Vincristine, Cisplatin, Cyclophosphamide and Etoposide. Doctors will collect patients' own blood and stem cells in order to put the stem cells back to boost patients' immune system in the next phase.

2. Consolidation Phase– 3 courses (21 days each course)

In each course, patients will receive Carboplatin, Thiotepa and receive their own blood stem cells in the Consolidation Phase.

At this point, some patients may need to be treated with radiation therapy in the trial.

3. Maintenance Phase– 12 courses (21 days each course)

Participants will take isotretinoin 2 times a day and SAHA every other day during the Maintenance Phase.

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 treatment affects the body.

WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-026?

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 a child stops taking SAHA.

Almost all of the drugs given in PBTC-026 can result in nausea, vomiting, hair loss, fatigue and other side effects. Isotretinoin may also cause joint pain, dry skin and sensitivity to light. SAHA when combined with chemotherapy may also reduce the numbers and characteristics of blood cells. Side effects are listed in more detail in the consent form for PBTC-026.

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-026?

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 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