PBTC-053 - A SUMMARY FOR PATIENTS AND FAMILIES

PBTC-053: Testing the Safety and Potential of CX-4945 to treat patients with recurrent medulloblastoma who may have surgery.

This is a brief summary of a clinical trial, a type of therapeutic research study. “You” refers to ‘you’ or ‘your child’ throughout this document. Clinical trials include only patients who choose to take part in the research study. Participation is entirely voluntary.

WHO MIGHT BE ELIGIBLE TO PARTICIPATE IN THIS STUDY?

Patients with SHH (Sonic hedgehog) medulloblastoma that is recurrent or progressive confirmed histologically.

This is a study of a drug called CX-4945. The study has 2 parts: the phase I study and the phase II study. The Phase I part of this study is only open to patients who are considered ‘skeletally-immature’. Skeletal immaturity is determined by taking an x-ray of your hand and wrist to compare your skeletal age to your actual age. This is called a ‘bone age’ x-ray. In order to enroll on this study, females must have a bone age that is <14 years of age. Males must have a bone age that is <16 years of age. The Phase II study is limited to older adolescents and adults. It is being conducted to test the safety of CX-4945 in older adolescents and adults and to see if the drug can shrink or stop tumors similar to yours from growing for a few months or longer. A small group of patients in each part may also take part in a surgical study. The surgical study will include both children and adults. You may take part in the Phase I or Phase II part of the study without taking part in the surgical study. Patients with extraneural disease only are also eligible.

WHAT IS THE PURPOSE OF THIS STUDY?

The primary objective of this study is to see if CX-4945 is safe and tolerable in patients. The surgical portion of the study is being done to find out how much of CX-4945 is found in tumor tissue removed during surgery and if the study drug works against a specific target found in your type of tumor. CX-4945 is considered experimental because it has not been proven to work in children with recurrent SHH medulloblastoma. We are using CX-4945 in this study because there is scientific reason to believe it may work against recurrent SHH medulloblastoma. However, we have not yet found a safe dose for children, or proven that the drug works for brain tumors. CX-4945 has been used in patients with other types of tumors.

WHAT IS INVOLVED IN THIS STUDY?

The study has 2 parts: the phase I study and the phase II study. The Phase I trial is being conducted to see if CX-4945 is safe and tolerable in children. The Phase II study is limited to older adolescents and adults. It is being conducted to test the safety of CX-4945 in older adolescents and adults and to see if the drug can shrink or stop tumors. The Phase II part of the trial will open first to test the safety of this formulation of CX-4945 in at least 3 skeletally mature patients. If no more than 1 toxicity is observed in this group, the Phase 1 part of the study will open for skeletally-immature subjects.

The first 3-6 study participants will receive the dose level that we think is suitable for skeletally-immature patients. If there are serious side effects, the dose level will not be increased any further. If CX-4945 does not cause serious side effects, the next group of 3-6 people in the study will get a higher dose. If serious side effects occur in this group, the dose will be lowered to the previous dose. A small group of patients in Phase I and Phase II may also take part in a surgical study. Participants in the surgical study will receive CX-4945 for 5-7 days before surgery and then continue after surgery if your doctor thinks it is safe. If you are in this group, you will take the dose of CX-4945 that did not have serious side effects before surgery. Some of the tumor tissue removed during your surgery will be used in the lab to look at the effect of CX-4945 on your type of tumor.

WHAT ARE THE RISKS OF PARTICIPATING IN THIS STUDY?

There is a risk that taking the study drug prior to surgery may not be effective or compromise the success of your surgery. There is also a risk that you could have side effects from the study drug. Some of the most common side effects that the study doctors know about are diarrhea, nausea, vomiting and fatigue. There may be some risks that the study doctors do not yet know about.

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 THIS STUDY?

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:

Ralph Salloum, MD

Cincinnati Children's Hospital Medical Center

3333 Burnett Avenue

Cincinnati, OH, 45229

Phone: 513-803-1126

Fax: 513-636-5349

Email: Ralph.Salloum@cchmc.org

OTHER INFORMATION IS AVAILABLE THROUGH

The National Cancer Institute's Cancer Information Service at 1-800-422-6237or TTY: 1-800-332-8615 or through the National Cancer Institute's websites www.Cancer.gov and www.cancer.gov/clinicaltrials.