Summary for Patients and Families

PBTC 059: Phase 1 Trial of Autologous HER2-specific CAR T cells in Pediatric Patients with Refractory or Recurrent Ependymoma

This research study is being done by the Pediatric Brain Tumor Consortium (PBTC) in collaboration with Texas Children's Hospital. The PBTC is a group dedicated to improving the treatment of childhood brain tumors. It comprises fifteen academic centers and children's hospitals in the United States and Canada.

The following is a summary of a clinical trial, a therapeutic research study. Clinical trials include only patients who choose or whose parents permit them to participate in the research study. Participation is entirely voluntary.

Who might be eligible to participate in PBTC-059?

Patient must have a diagnosis of HER2-positive ependymoma that is recurrent or progressive. Patient must have received standard treatment such as a surgical resection followed by local adjuvant radiation therapy before enrollment. Patients that are known to be HIV-positive are ineligible. Patients must be 1- 21 years of age at the time of screening consent. Patients will undergo clinical evaluations to confirm adequate organ and bone marrow function prior to starting the trial.

Why is PBTC-059 being undertaken?

This study is to determine the safety of a new treatment for ependymoma, for which effective therapy is still lacking. This new treatment involves intravenous infusion of HER2-specific CAR T cells in patients with progressive or recurrent HER2-ependymoma. Patients will receive up to three intravenous infusions of HER2-specific CAR T cells.

What is involved in this study?

If you decide to participate in this study, you will receive treatment in two parts; 1) lymphodepletion, which involves treatment with fludarabine and cyclophosphamide in an inpatient or outpatient setting, and 2) HER2 CAR T cell infusion in an inpatient setting. Blood samples will be collected before and after these treatments. During these treatments, patients will receive other medications for supportive care. Patients who receive up to 3 courses of CAR T cells may be on treatment for up to 9 months. There is a follow up period for up to 15 years after the last HER2 CAR T cell infusion once per year.

What are the risks of participating in PBTC-059?

There is a risk that patients may experience side effects from chemotherapy and the following HER2 CAR T cell administration. Some of the most common side effects the study doctors know about are fever, chills, tiredness, body aches, increased heart rate, headache, breathing difficulties, nausea, vomiting, allergic reaction, hair loss, and skin rashes. There is a possibility for organ damage, neurological symptoms, and the occurrence of other cancers. 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. In the event of adverse effects, the healthcare team may modify the treatment and/or incorporate additional medical tests to monitor adverse effects. Doctors will notify parents and patients immediately of any critical 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:

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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 www.Cancer.gov and www.cancer.gov/clinicaltrials.