

PBTC-014 - A SUMMARY FOR PATIENTS AND FAMILIES

TITLE: Phase I/II Trial of R115777 and XRT in Pediatric Patients with Newly Diagnosed Non-Disseminated Intrinsic Diffuse Brainstem Gliomas

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-014?

Children between the ages of 3 and 21 years of age who have a newly diagnosed brain stem glioma, which has not spread within the brain stem, may be eligible to participate in PBTC-014.

Children who have already received chemotherapy, radiation therapy or bone marrow transplant are not eligible for this trial. Children who may be taking certain kinds of anti-seizure medications may also not be eligible to participate. Patients who are pregnant or breast-feeding cannot participate in this clinical trial.

Patients will need medical tests to assess whether they can participate in PBTC-014. 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 46 children from all over the U.S. will take part in PBTC-014.

WHY IS PBTC-014 BEING DONE?

Current treatment for highly malignant gliomas in children is radiation therapy, and survival rates are poor. PBTC-014 tests a new investigational drug in combination with radiation therapy. This new drug is called R115777, also known as Zarnestra or tipifarnib.

In studies with animals, R115777 stops certain abnormal cells from growing, while causing little harm to normal cells. R115777 belongs to a group of compounds called “anti-farnesylation” agents. PBTC-014 will determine what effects (good or bad) R115777 has on study participants and on their brain stem gliomas when given with radiation therapy.

Doctors and researchers are conducting PBTC-014 in the hopes of finding a more effective treatment for brain stem gliomas in children.

WHAT IS INVOLVED IN PBTC-014?

Study participants will swallow R115777 in a tablet and/or capsule twice a day. The tablet and/or capsule should be taken with a meal or a snack. R115777 will start around the time that radiation therapy will start. The tablets and/or capsules should be taken every day during the course of radiation therapy. Radiation therapy will last about 6 weeks. After radiation therapy

ends, participants will take R115777 for up to two years according to an alternating weekly schedule specified in the clinical trial.

WHAT ARE THE RISKS OF PARTICIPATING IN PBTC-014?

Study participants will be watched carefully for any side effects or other problems. Some of the side effects that may occur include low blood cell counts, fever, fatigue or drowsiness, nausea, vomiting, muscle weakness, sleeplessness and stomach pain.

There may be unknown risks and discomforts involved in participating in this or any clinical trial. 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-014?

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:

Daphne Haas-Kogan, MD

University of California, San Francisco
1600 Divisadero St
San Francisco, CA 94143-1708
Phone: (415) 353-7175
Email: hkogan@radonc17.ucsf.edu

Anuradha Banerjee, MD, MPH

University of California, San Francisco
Div. of Neuro-oncology
Department of Neurosurgery
400 Parnassus Avenue
Room A808, Box 0372
San Francisco, CA 94143-0372
Phone: (415) 353-2966
Email: Banerjee@neurosurg.ucsf.edu

OTHER INFORMATION IS AVAILABLE THROUGH

The NCI's Cancer Information Service at 1-800-4-CANCER (1-800-422-6237) or TTY: 1-800-332-8615 or visit the NCI's websites

- <http://cancer.gov>
- <http://cancer.gov/clinicaltrials>
- <http://cancer.gov/cancerinfo>