



News Release

STANFORD UNIVERSITY MEDICAL CENTER

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STANFORD/PACKARD'S STUDY OF VACCINE FOR CHILDREN'S BRAIN CANCER SEEKS PARTICIPANTS

STANFORD, Calif. – Scientists at the Stanford University School of Medicine and Lucile Packard Children's Hospital are starting a phase-1 clinical trial of a vaccine-based treatment for the deadliest childhood cancer, a rare brain tumor called Diffuse Intrinsic Pontine Glioma. Today, just 1 percent of DIPG patients survive five years past diagnosis. No cure exists.

"Thirty years of numerous clinical trials have all failed," said Gordon Li, MD, who is an acting assistant professor of neurosurgery at Stanford and one of the study's co-investigators. The tumor, seen mostly in school-aged children, is impossible to remove surgically because it grows tangled into a region of the brain stem essential for life. It does not respond to chemotherapy, and radiation gives only temporary remission.



Li and his colleagues are testing an approach never tried before for DIPG: They are giving an anti-cancer vaccine to try to make the patient's own body attack the tumor. In the new trial, funded by a \$1.5 million grant from the National Institutes of Health, patients will receive regular doses of a therapeutic vaccine against EGFRvIII, a cell marker that Li recently discovered is found on about half of DIPG tumors. Unlike prophylactic vaccines given to prevent disease, the goal of this treatment is to alert the body's immune system that disease is already present. The researchers hope the vaccine will cause the body to produce immune cells that recognize and destroy tumor cells carrying the EGFRvIII marker.

"If a vaccine trial works, it could advance the concept of treatment for all types of tumors with immune therapy," Li said. Immune therapies have been tested with mixed success in a few adult cancers, but only one, a prostate cancer treatment, has been approved by the U.S. Food and Drug Administration. The EGFRvIII vaccine is promising because its target - a molecular flag on the exterior of cancer cells - is a very different molecular shape than the markers on healthy cells, the researchers said.

The researchers plan to enroll 15 children with recent DIPG diagnoses for the trial.

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"The study subjects will be getting conventional radiation therapy and then a series of monthly vaccinations to mount an immune response against the tumor," said Paul Fisher, MD, a pediatric oncologist at Packard Children's who is a principal investigator on the study. Because it is a phase-1 trial, the study's main purpose is to establish the vaccine's safety, but the vaccine's efficacy will also be monitored. Patients will receive up to 12 months of vaccine injections and will be monitored with MRIs, immunologic exams and physical exams. After the 12-month vaccine period ends, they will be followed by monitoring for as long as they remain alive.

More information about the trial is available online at <http://med.stanford.edu/clinicaltrials/detail.do?studyId=4642>, by e-mailing neuroonc@lpch.org or by calling Fisher at (650) 497-8953.

Albert Wong, MD, professor of neurosurgery at Stanford, is leading the study with Fisher. Wong is one of the patent holders on the discovery of the EGFRvIII receptor and for using EGFRvIII as an anti-tumor vaccine. He also holds stock in Celldex, the company supplying vaccine for the trial.

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Stanford University Medical Center integrates research, medical education and patient care at its three institutions – Stanford University School of Medicine, Stanford Hospital & Clinics and Lucile Packard Children's Hospital at Stanford. For more information, please visit the Web site of the medical center's Office of Communication & Public Affairs at <http://mednews.stanford.edu>.