CEDARS-SINAI NEWS

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Update: 50 Percent of Patients in Cedars-Sinai Brain Cancer Study Alive After Five Years

With Standard Care, Median Length of Survival is 15 Months After Diagnosis of Glioblastoma Multiforme – and Only 10 Percent Survive More Than 5 Years

LOS ANGELES (NOV. 24, 2013) – Eight of 16 patients participating in a study of an experimental immune system therapy directed against the most aggressive malignant brain tumors – glioblastoma multiforme – survived longer than five years after diagnosis, according to Cedars-Sinai researchers, who presented findings Nov. 23 at the Fourth Quadrennial Meeting of the World Federation of Neuro-Oncology.

Seven of the 16 participants still are living, with length of survival ranging from 60.7 to 82.7 months after diagnosis. Six of the patients also were "progression free" for more than five years, meaning the tumors did not return or require more treatment during that time. Four participants still remain free of disease with good quality of life at lengths ranging from 65.1 to 82.7 months following diagnosis. One patient who remained free of brain cancer for five years died of leukemia.

The original clinical trial – a Phase I study designed to evaluate safety – included 16 patients with glioblastoma multiforme enrolled between May 2007 and January 2010 by researchers at Cedars-Sinai's Johnnie L. Cochran, Jr. Brain Tumor Center.

Results published in January at the end of the study showed median overall survival of 38.4 months. Typically, when tumor-removal surgery is followed by standard care, which includes radiation and chemotherapy, median length of survival is about 15 months. Median progression-free survival – the time from treatment to tumor recurrence – was 16.9 months at study's end. With standard care, the median is about seven months.

The experimental treatment consists of a vaccine, ICT-107, intended to alert the immune system to the existence of cancer cells and activate a tumor-killing response. It targets six antigens involved in the development of glioblastoma cells.

According to information presented at the scientific meetings, all eight long-term survivors had tumors with at least five antigens, 75 percent had tumors with all six, and 100 percent had tumors with at least four antigens associated with cancer stem cells – cancer-originating cells that appear to enable tumors to resist radiation and chemotherapy and even regenerate after treatment.

(more)

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"Our findings suggest that targeting antigens that are highly expressed by cancer stem cells may be a viable strategy for treating patients who have glioblastomas. Long-term remission of disease in this group of patients was correlated with the expression of cancer stem cell tumor-associated antigens," said Surasak Phuphanich, MD, director of the Neuro-Oncology Program at the Cochran Brain Tumor Center and professor of neurology with Cedars-Sinai's Department of Neurosurgery and Department of Neurology.

Based on results of the Phase I study, the ICT-107 vaccine entered a Phase II multicenter, randomized, placebo-controlled trial in 2011.

The vaccine is based on dendritic cells, the immune system's most powerful antigen-presenting cells – those responsible for helping the immune system recognize invaders. They are derived from white blood cells taken from each participating patient in a routine blood draw. In the laboratory, the cells are cultured with synthetic peptides of the six antigens – essentially training the dendritic cells to recognize the tumor antigens as targets. When the "new" dendritic cells in the vaccine are injected under the patient's skin, they are intended to seek and destroy lingering tumor cells. Vaccine is administered three times at two-week intervals after standard radiation and chemotherapy.

Phuphanich is first author of an abstract presented at the scientific meetings' poster session from 5 to 7 p.m. PST Nov. 23.

ICT-107 is a product of the biotechnology company ImmunoCellular Therapeutics, Ltd. Cedars-Sinai owns equity in the company, and certain rights in the dendritic cell vaccine technology and corresponding intellectual property have been exclusively licensed by Cedars-Sinai to ImmunoCellular Therapeutics, including rights associated with ICT-107, the vaccine investigated in this clinical study.

Several members of the research and presentation team have ties to the company. Abstract co-author Keith Black, MD, a Cedars-Sinai physician, owns stock in the company. Senior author John Yu, MD, a Cedars-Sinai physician, owns stock in the company and is its founder, chief scientific officer and chair of the board of directors. James Bender, PhD, MPH, a co-author, is Immunocellular Therapeutics' vice president for product development and manufacturing. Elma Hawkins, a co-author, also is identified with Immunocellular.

Co-authors who do not have relationships with the company include: Surasak Phuphanich, MD, PhD, first author; Christopher Wheeler, PhD; Jeremy Rudnick, MD; Jethro Hu, MD; Mia Mazer; Hong Q. Wang; Miriam Nuno; Cherry Sanchez; Xuemo Fan; Jianfel Ji; and Ray Chu, MD.

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