Immunotherapy effective against neuroblastoma in children

May 14 2009

A phase III study has shown that adding an antibody-based therapy that harnesses the body's immune system resulted in a 20 percent increase in the number of children living disease-free for at least two years with neuroblastoma. Neuroblastoma, a hard-to-treat cancer arising from nervous system cells, is responsible for 15 percent of cancer-related deaths in children. The researchers reported their findings - the first to show that immunotherapy could be effective against childhood cancer - online May 14, 2009 on the American Society of Clinical Oncology website in advance of presentation June 2.

"This establishes a new standard of care for a traditionally very difficult cancer in children," said lead author Alice Yu, MD, PhD, professor of pediatric hematology/oncology at the University of California, San Diego School of Medicine and the Moores UCSD Cancer Center. "High-risk neuroblastoma has always been a frustrating cancer to treat because, despite aggressive therapy, it has a high relapse rate."

The therapy targets a specific glycan (a complex sugar chain found on the surface of cells) on neuroblastoma cells called GD2, which inhibit the immune system from killing cancer cells. The antibody - ch14.18 - binds to this glycan, enabling various types of immune cells to attack the cancer.

Neuroblastoma - in which the cancer cells arise from nerve cells in the neck, chest, or abdomen - is the most common cancer diagnosed in the first year of life. Approximately 650 new cases of neuroblastoma are

diagnosed in this country every year, and about 40 percent of patients have high-risk neuroblastoma. These high-risk patients are usually treated with surgery, intensive chemotherapy with stem cell rescue (in which patients' adult stem cells, removed before treatment, are returned after chemotherapy to restore the blood and immune system), and radiation therapy. Still, only 30 percent of patients survive.

Yu and her colleagues compared both the percentage of patients who were still alive without experiencing a recurrence after two years as well as overall survival in two groups of 113 patients each. Patients began the trial when they were newly diagnosed with high-risk neuroblastoma. After conventional treatment with surgery, chemotherapy, stem cell rescue and radiotherapy, one group was given the standard treatment (retinoic acid) plus immunotherapy (the antibody plus immune-boosting substances), while 113 similar patients received the standard treatment alone.

After two years, 66 percent of individuals in the immunotherapy group were living free of cancer compared to 46 percent in the standard treatment group. Overall survival improved significantly as well. The trial patient randomization was halted early because of the benefit seen, and all patients enrolled in the trial will receive immunotherapy plus standard treatment.

Yu noted that the two-year mark is especially important because past trials have shown that those neuroblastoma patients who live without disease for two years after a stem cell transplant will most likely be cured.

"This is the first time in many years that we have been able to improve the 'cure rate' for neuroblastoma patients," she said. "This new therapy can help us improve care and perhaps offer new hope to many patients and families."

Yu and her team conducted the early phase I and phase II trials at the General Clinical Research Center at UC San Diego Medical Center.

Source: University of California - San Diego (news : web)