MedicalXpress

New CAR T case study shows promise in acute myeloid leukemia

May 9 2018

Chimeric Antigen Receptor T-cell therapy, also known as CAR T therapy, was named the biggest research breakthrough of 2017 by the American Society of Clinical Oncology. The personal gene therapy utilizes a patient's own immune cells to fight cancer. The Food and Drug Administration has approved CAR T therapy products for adults with diffuse large B-cell lymphoma and pediatric and young adults suffering from acute lymphoblastic leukemia. Now, researchers at Moffitt Cancer Center are working to expand this revolutionary therapy to other cancers.

One possible disease that could benefit is acute myeloid leukemia (AML), the most common acute leukemia affecting adults. More than half of AML patients go into remission after chemotherapy. However, many relapse because residual leukemia cells can evade chemotherapy and the immune system. A new phase 1 trial called the THINK (THerapeutic Immunotherapy with NKG2D) study is investigating Celyad's new CAR T therapy CYAD-01 which genetically modifies immune cell to express a natural killer receptor that targets leukemia tumors.

According to a case study from trial published online ahead of print in the journal *Haematologica*, a patient has remained cancer free for nine months after being treated with CYAD-01, followed by a bone marrow transplant.

"It is important to note this is the first time CAR T has induced a remission in AML," said case study author David Sallman, M.D.,

assistant member of the Department of Malignant Hematology at Moffitt. "It is also the first time CAR T has been effective without the need of preconditioning chemotherapy."

For CAR T treatment, T cells are removed from a patient's blood and sent to a lab where the cells are genetically modified to better enable them to identify and attack cancer cells. Once the new cells are received, patients typically receive preconditioning chemotherapy to deplete their immune system to make room for the new cells. In the THINK study, no preconditioning chemotherapy was necessary.

"Our case study shows that CAR T therapy is a viable option for AML patients," added Sallman. "We now need to take what we have learned in this initial study and expand the trial."

More information: David A. Sallman et al, NKG2D-based chimeric antigen receptor therapy induced remission in a relapsed/refractory acute myeloid leukemia patient., *Haematologica* (2018). DOI: 10.3324/haematol.2017.186742

Provided by H. Lee Moffitt Cancer Center & Research Institute