MedicalXpress

US regulators fast-track novel leukemia therapy

July 7 2014

US regulators on Monday put an experimental immunotherapy agent on the fast track to market approval, after 89 percent of leukemia patients in early trials saw their cancers disappear.

The personalized immunotherapy known as CTL019 was developed by the University of Pennsylvania and was designated a "breakthrough therapy" by the US Food and Drug Administration.

That means the experimental therapy will benefit from a speedier than average review process and will get extra attention from the FDA toward development for market.

It is the first cancer immunotherapy to receive the breakthrough designation, and only the fifth biologic agent so far.

The approach works by extracting a patient's T-cells, then genetically programming them in the lab to target cancer cells that produce a protein called CD19.

The altered T-cells are then re-injected into the patient's body, where they multiply and attack cancer.

Researchers reported last year that of 27 patients, including 22 children and five adults, with acute lymphoblastic leukemia, 89 percent had a complete response to the therapy, meaning their cancer became undetectable.

The first child to receive the treatment, Emily Whitehead, in May marked two years of being in remission, and the first adult patient has been in remission for one year.

"Our early findings reveal tremendous promise for a desperate group of patients, many of whom have been able to return to their normal lives at school and work after receiving this new, personalized immunotherapy," said the Penn research team's leader, Carl June.

The university in 2012 teamed up with pharmaceutical company Novartis to develop and license personalized chimeric antigen receptor (CAR) T cell therapies for the treatment of cancers.

In addition to the ongoing trials for acute lymphoblastic leukemia, trials using CTL019 began in the summer of 2010 in patients with relapsed and refractory chronic lymphocytic leukemia.

It is also being tried in patients with non-Hodgkin lymphoma and myeloma.

More research is also under way into CAR therapies for mesothelioma, ovarian, breast and pancreatic cancers.