First US gene therapy nears: CAR-T leukemia treatment gets nod from FDA panel

Novartis AG's pioneering new cancer drug won enthusiastic support from a federal advisory panel on [July 12], paving the way for approval of the first U.S. gene therapy.

The panel unanimously recommended that the Food and Drug Administration approve the drug, tisagenlecleucel, for patients ages 3 to 25 with relapsed B-cell acute lymphoblastic leukemia (ALL), the most common form of U.S childhood cancer.

The drug uses a new technology known as CAR-T, or chimeric antigen receptor T-cell therapy, which harnesses the body's own immune cells to recognize and attack malignant cells.

In a clinical trial, 83 percent of patients who had relapsed or failed chemotherapy achieved complete or partial remission three months post infusion. After one year 79 percent of patients were still alive.

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The FDA is not obliged to follow the recommendations of its advisers but typically does so. The agency is expected to rule on the drug by the end of September.

Approval of tisagenlecleucel would…advance a technique scientists have been attempting to perfect for decades and help lift the entire field of cell therapy.

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"This will be a historic approval," said Brad Loncar, chief executive of Loncar Investments which runs the Loncar Cancer Immunotherapy ETF. "As an investor I've never seen anything like it. It's an entirely new way of treating cancer."

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: FDA panel backs Novartis' pioneering new cancer gene therapy