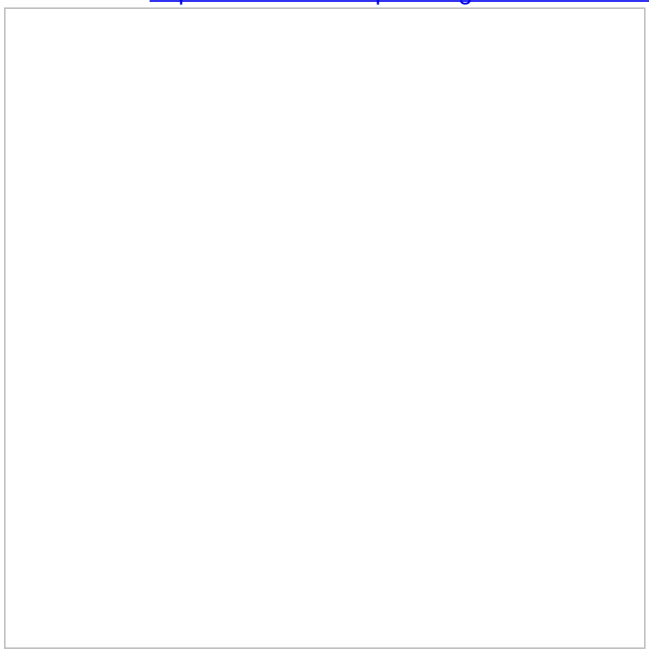
Gene Therapy Approved For Cancer In U.S.



On August 30, 2017, the FDA officially approved the cancer treatment Kymriah, the first gene therapy legally available in the United States. It will be used to treat B-cell precursor acute lymphoblastic leukemia (ALL) in patients below the age of 25.

In this form of leukemia, patients produce abnormal B cells, a type of white blood cell. White blood cells are critical to the immune system. Since the immune system fights infection, the abnormal cells can be lethal if not treated effectively. An estimated 15-20% of patients do not respond to initial treatment or respond but then have a recurrence of their disease. This group of patients is now eligible for Kymriah.

In this gene therapy, normal white blood cells are removed from the body and sent to the laboratory, where they are genetically altered. The modification arms the cells with a protein called a chimeric antigen receptor (CAR). The CAR acts like a magnet, which is able to bind to the leukemia cells. The altered white blood cells are then released back into the patient, where they can bind to, and kill, cancer cells. The results with CAR-based treatments are very exciting, with many patients showing no evidence of disease after the treatments.

Source
https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm574058.htm
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