First gene therapy approved to treat leukemia
by Melissa Jenco, News Content Editor

The Food and Drug Administration (FDA) has approved the first gene therapy to treat children and young adults with leukemia.

Kymriah from Novartis Pharmaceuticals Corp. is a chimeric antigen receptor T cell therapy in which a patient's T cells are collected and genetically modified to kill leukemia cells, according to the FDA. They then are infused back into the patient to fight the cancer cells.

"We're entering a new frontier in medical innovation with the ability to reprogram a patient's own cells to attack a deadly cancer," FDA Commissioner Scott Gottlieb, M.D. said in a news release. "New technologies such as gene and cell therapies hold out the potential to transform medicine and create an inflection point in our ability to treat and even cure many intractable illnesses."

Each year, about 3,100 patients ages 20 and younger are diagnosed with acute lymphoblastic leukemia (ALL), a cancer of the bone marrow and blood.

Kymriah is intended for patients with B-cell precursor ALL in which the cancer returned after treatment or did not respond. The therapy received priority review and breakthrough therapy designations from the FDA and was approved for patients up to 25 years. About 83% of the 63 patients in one clinical trial went into remission with Kymriah therapy.

"A person who has had a relapse in their leukemia, it's often very difficult to get them into remission or keep them in remission after relapse," said Jeffrey Hord, M.D., FAAP, past chair of the AAP Section on Hematology and Oncology and director of the Division of Hematology-Oncology at Akron Children's Hospital. "... (Kymriah) has got a lot of toxicity with it, but with a single infusion these patients have been kept in remission from their leukemia for very long periods of time. It's pretty remarkable."

Kymriah contains a boxed warning for cytokine release syndrome and has the potential for other severe side effects, including serious infections, low blood pressure, acute kidney injury, fever and decreased oxygen. However, Dr. Hord noted patients who qualify to use it typically are out of other options.

The therapy does not replace chemotherapy as the frontline treatment for most children with ALL.

Hospitals and clinics will need to be specially certified to use Kymriah. Novartis said it is working with the Centers for Medicare & Medicaid Services on pricing and access issues.