In August 2017, the FDA approved the first adoptive cell immunotherapy, also known as CAR T-cell therapy, and the first gene therapy for cancer, tisagenlecleucel. This double first approval stems from decades of research on how to train the patient’s own immune cells to fight cancer. Even more important than the historic significance of this achievement is the medical need this unique new therapy is poised to fill. Tisagenlecleucel may be the first treatment to truly turn the tables on recurrent pediatric acute lymphoblastic leukemia (ALL), one of the most common cancers in children. In a clinical trial, in four of five patients, cancer went into remission after the treatment, which was custom prepared in the laboratory from the patients’ own blood cells.

In October 2017, the FDA approved the second CAR T-cell therapy, axicabtagene ciloleucel, to treat adults with certain types of lymphoma. Other CAR T-cell therapies seem promising in clinical trials of people with multiple myeloma. CAR T-cell therapy represents an exciting innovation that has the potential to transform cancer care. It also raises the ongoing issue of cost and reminds us that, as a community, we need to find solutions that will assure that every patient with cancer has access to the care they need (see Advance of the Year: Adoptive Cell Immunotherapy).

The other historic first among FDA approvals in 2017 marks a milestone in precision oncology. The immune checkpoint inhibitor pembrolizumab became the first cancer treatment to receive a tumor-agnostic indication.

It received accelerated approval to treat any type of solid tumor that has mismatch repair deficiency, a defect that undermines the cell’s ability to repair DNA damage. This approval provides patients with a wide range of different cancers an effective way to control the disease.

Another promising treatment, larotrectinib, which homes in on a different, rare genomic abnormality in the tumor known as tropomyosin receptor kinase (TRK) gene fusion, also seems to work across tumor types and in both adults and children. Larotrectinib has the potential to become the first tumor-agnostic targeted therapy for cancer.

Meanwhile, fundamental cancer biology research is uncovering new molecular pathways that are being explored as potential therapeutic targets. In 2017 alone, the FDA approved more than 13 new targeted medicines for people with leukemia, and multiple myeloma, as well as ovarian, breast, and lung cancer.

ASCO estimates that immune checkpoint inhibitors would save 250,000 years of life if all US patients with advanced lung cancer for whom checkpoint inhibitors are currently indicated receive the treatment.

In addition, one in four patients with newly diagnosed cancer and one in 10 with previously treated disease may live well beyond 5 years after treatment initiation. The estimates were based on an analysis of clinical trial results and cancer incidence and population data conducted by the ASCO Center for Research and Analytics (CENTRA).