Drug Approvals Mark New Era in Treating Acute Leukemia in Adults

Oncologists are excited about the increased options available to patients, and about the promise of even more new therapies to come

June 8, 2018 By Dana-Farber Cancer Institute

The Food and Drug Administration (FDA) issued 18 approvals for blood cancer therapies in 2017, and oncologists are excited about the increased options available to patients, and about the promise of even more new therapies to come. Although not all of the approvals were for novel drugs—some were for new uses of already-approved agents—they collectively mark a new era in the treatment of adults with these diseases, scientists say.

The impact of the approvals is especially notable in the two forms of acute leukemia—acute lymphoblastic leukemia (ALL) and acute myeloid leukemia (AML). In the case of AML, which accounts for about 75 percent of acute leukemias in adults, 2017 brought the approval of four new therapies—a striking achievement considering that no new drugs had been approved for the disease in the previous 40 years. Three new treatments also received FDA approval for ALL.

AML

The four drugs approved for adult AML in 2017 were:

- Midostaurin for patients whose AML carries a mutation in FLT3 gene;
- Enasidenib for patients with relapsed or drug resistant AML that carries an IDH2 gene mutation;
- CPX-351, which delivers two chemotherapy agents for patients newly diagnosed with therapy-related AML or AML with myelodysplasia-related changes;
- Gemtuzumab ozogamicin, an immunotherapy drug for patients newly diagnosed with AML that produces a protein called CD33.

These approvals are the leading edge of a new wave of treatments for AML. This year, we're expecting to learn the results of a clinical trial comparing quizartinib, a new agent that targets the FLT3 protein, with standard chemotherapy in patients with FLT3-mutated AML," says Richard Stone, MD, Dana-Farber chief of staff and director of the Institute's Program in Adult Leukemia.

“We also expect to see the findings of a trial comparing gilteritinib, which targets both FLT3 mutation subtypes, with standard chemotherapy for these patients.”

“We’re also excited about the use of the drug venetoclax in patients with AML,” he continues. “Venetoclax inhibits BCL2, a protein that prevents cells from dying in response to stress such as radiation or chemotherapy. There have been some encouraging reports about the combination of venetoclax and chemotherapy in older patients whose health might otherwise make them unfit for chemotherapy alone.”

ALL

In 2017, three drugs received FDA approval for treatment of patients with ALL that has returned or not responded to previous treatments:

- Blinatumomab, an immunotherapy agent for patients with relapsed, treatment-resistant B-cell ALL;
- Inotuzumab, a drug that unites an antibody with a cancer-fighting antibiotic, for patients with B-cell ALL;
- Tisangenlecleucel, a CAR T-cell therapy made from specially engineered versions of a patient’s own T cells. It has been approved for patients under age 26.

Many of the new approaches to treating adults with ALL were inspired by the success of approaches used in treating children with the disease, notes Daniel DeAngelo, MD, PhD, director of Clinical and Translational Research in Dana-Farber’s Adult Leukemia Program.

“With the approval of these new agents in patients with relapsed or resistant ALL, the next step will be to test them in trials for patients who are newly diagnosed with the disease,” he remarks.

This article was originally published on May 26, 2018, by Dana-Farber Cancer Institute. It is republished with permission.
