FDA Approves Rytelo, the First Telomerase Inhibitor, for Myelodysplastic Syndromes

People who received imetelstat were able to go longer without needing red blood cell transfusions.

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On June 6, the Food and Drug Administration (FDA) approved Rytelo (imetelstat) for certain patients with myelodysplastic syndromes (MDS), a group of malignant blood disorders in which immature blood cell precursors in the bone marrow don't mature properly. MDS can cause anemia and may progress to leukemia.

Rytelo, from Geron Corporation, is the first approved oligonucleotide telomerase inhibitor. Telomeres are protective caps at the ends of chromosomes that naturally shorten each time a cell divides, eventually leading to cell death. Rytelo blocks the activity of telomerase, an enzyme that rebuilds telomeres and allows uncontrolled cell division.

The FDA approved Rytelo for adults with low- to intermediate-risk MDS with transfusion-dependent anemia who require four or more units of red blood cell units over eight weeks and who do not respond to or are ineligible for erythropoiesis-stimulating medications that promote red blood cell production.

The approval is supported by findings from the Phase III IMerge trial (NCT02598661), which enrolled 178 people with MDS. Participants were randomly assigned to receive an IV infusion of Rytelo or a placebo in 28-day cycles until they experienced disease progression or unacceptable toxicity. Results were published in The Lancet in January.

After a median follow-up time of about 18 months, people in the Rytelo group were significantly less likely to need red blood cell transfusions. About 40% of patients taking Rytelo were able to go without transfusions for at least eight consecutive weeks, compared with 15% in the placebo group; 28% and 3%, respectively, had transfusion independence for at least 24 consecutive weeks. The median duration of transfusion dependence for eight-week and 24-week responders was approximately one year and 1.5 years, respectively.

Treatment was generally safe. The most common severe (grade 3-4) adverse events in the Rytelo

group were decreased white blood cell counts (including neutropenia) and decreased platelet count, which can lead to slow blood clotting. In most cases, these were manageable and temporary.

"For patients with lower-risk MDS and anemia who are transfusion dependent, we have very few options today and often cycle through available therapies, making the approval of Rytelo potentially practice changing for us," IMerge investigator Rami Komrokji, MD, of Moffitt Cancer Center, said in a news release. "The treatment goal for patients with lower-risk MDS and anemia is transfusion-independence, and before today, this wasn't possible for many patients."

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