FDA Approves Novel Treatment for Acute Myeloid Leukemia

Damaon Runyon researcher's work helped expand the use of Revuforj (revumenib) for certain leukemia patients with NPM1 mutations

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Acute myeloid leukemia (AML) is diagnosed in more than 20,000 people in the U.S. each year, and the five-year survival rate remains around 30%. For patients whose disease is driven by mutations in the gene NPM1, the most common mutation in adult AML, treatment options have long been limited.

But after decades of research, a novel treatment now offers hope for these patients. In October 2025, the Food and Drug Administration approved a drug known as revumenib for adults and children with NPM1-mutated AML. Revumenib had previously been approved for AML patients with KMT2A mutations, which are relatively rare. With this expanded approval, the drug can now reach up to 40% of AML patients.

But the path to approval began in 2016, when former Damon Runyon Clinical Investigator Scott Armstrong, MD, PhD, and his lab at Dana-Farber Cancer Institute observed that NPM1-mutated AML cells depend on a protein called menin for survival. Their work confirmed the hypothesis and set the stage for clinical testing of revumenib, a menin inhibitor, for treatment of this AML subtype.

"It is personally satisfying to see that the hard work of our scientists has led not only to a novel approach to AML therapy but also to its availability to many more patients," Dr. Armstrong told his institution.

Trials of multiple drug combinations with revumenib are now underway, he explained, as these combinations are likely to be even more effective than revumenib alone. Read the full press release here.

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