

Drug shows the potential for long-term relief from a type of chronic leukemia

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Video Abstract

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Abstract

Researchers from The University of Texas MD Anderson Cancer Center have good news for patients with myelofibrosis, a serious cancer that causes blood cells to grow uncontrollably, leading to severe scarring in bone marrow. In concluding a clinical trial started in 2009, they've found a safe and effective long-term treatment option for patients with the disease. Overall, the trial results showed that treatment with ruxolitinib not only reduced some of the more severe symptoms of myelofibrosis but also significantly improved survival. The trial, called COMFORT-I, took place at 89 sites across Australia, Canada, and the USA and looked at about 300 patients with the worst types of myelofibrosis. Because the disease interferes with blood cell production, these patients experience symptoms such as severe weakness and fatigue, anemia and spleen enlargement. They also have shortened survival. In the trial, the patients were randomized to receive either ruxolitinib or a placebo. Three-year results showed that the drug reduced spleen volume back to normal levels and lengthened survival, but the study was still ongoing. Now, a final analysis performed at 5 years suggests those good results are even longer-lasting. Spleen volumes remained normal over this time frame, and the patients treated with ruxolitinib were 30% less likely to die than those who received placebo. Additionally, some side effects that were more common early in the trial either disappeared or stabilized to manageable levels. Ruxolitinib is an inhibitor of the JAK cell signaling pathway, although the exact mechanism by which it improves different patient outcomes is still unknown. Collectively, the 5-year results show that ruxolitinib is a good long-term option for many patients with severe myelofibrosis. As physicians learn more about optimal dosing, it may be possible to further minimize adverse side effects and produce even better outcomes.