

# New protein-targeting drug shows promise in early trial for patients with high-risk CLL

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A new oral targeted drug, idelalisib (GS-1101), has the potential to stave off the need for additional treatments for relapsed or treatment-resistant chronic lymphocytic leukemia (CLL), according to a study led in part by Dana-Farber Cancer Institute investigators. In a phase 1 clinical trial, the drug produced rapid and long-lasting tumor shrinkage in about two-thirds of patients, stalling disease progression for 17 months, on average. The activity of the drug is noteworthy, given that the patients had an average of five prior therapies.

CLL, which is often diagnosed through a routine blood test, is a slow-growing cancer and many patients do not require treatment until they start having symptoms. The vast majority of patients with CLL experience a relapse at some point after initial treatment with a combination of chemotherapy and [antibody therapy](#). About 20 percent have so-called refractory disease, meaning they either relapse quickly (within six months) or do not respond to initial treatment at all. Patients in both categories are in need of better treatments, as the currently approved options have limited success.

Idelalisib is the first [drug](#) that selectively blocks [PI3K](#)-delta, a subtype of PI3K proteins that is overactive specifically in [B cell lymphoma](#), and is critical for [tumor growth](#). This is the first study of a PI3K-delta inhibitor in patients with CLL.

"We are reaching a point in the treatment of CLL where we have multiple agents in development that are very effective, and drugs like

idelalisib are probably going to change the landscape of the disease in the next few years," said the study's lead author Jennifer R. Brown, MD, PhD, director of the [Chronic Lymphocytic Leukemia](#) Center at Dana-Farber.

"While this research is still early and ongoing, we hope this drug, along with others like it, will lead to prolonged survival and eventually help turn CLL into a condition that is treated like [high blood pressure](#), where a patient can take a couple of pills every day," Brown added. "In the shorter term, these drugs may also provide an alternative to chemotherapy in elderly patients who tend not to tolerate chemotherapy well."

The clinical trial included 54 patients with refractory or relapsed CLL who received idelalisib. The patients' cancer had worsened despite having been treated with a median of five prior therapies. Once idelalisib treatment began, tumor reduction with a decrease in symptoms was rapid, generally occurring within two months, and was observed in about two-thirds of patients.

The 17-month average period in which the disease was held in check was better than the six- to 12-month benefit that might typically be expected for a sixth-line therapy. Patients who benefited from the drug were offered the opportunity to continue treatment on an extension study.

The researchers reported that side effects were manageable; only seven percent of patients discontinued treatment due to treatment-related effects.

Provided by Dana-Farber Cancer Institute

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