

Immunotherapy strategy could be beneficial for relapsed acute myeloid leukemia

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University of North Carolina Lineberger Comprehensive Cancer Center researchers report that pairing an immunotherapy drug with chemotherapy proved beneficial for some patients with acute myeloid leukemia whose disease did not respond to standard treatment or had relapsed.

Seven out of 18 [patients](#) with relapsed or persistent AML, or nearly 39 percent, had a complete remission following [treatment](#) with pembrolizumab, a checkpoint inhibitor, and high-dose [chemotherapy](#). The researchers will present their preliminary results from the ongoing phase II trial at the 59th American Society of Hematology Annual Meeting in Atlanta on Saturday, Dec. 9.

"I'm optimistic about the future for immunotherapy, particularly checkpoint inhibitors, in AML, and I think the future direction will be finding the right setting, and finding the right treatment sequence, in which to use them for this disease," said UNC Lineberger's Joshua Zeidner, MD, assistant professor in the UNC School of Medicine.

Researchers are studying whether they can achieve a complete remission in 40 percent of patients using a combination of chemotherapy followed by pembrolizumab. In an early stage of the trial, they observed responses in enough patients to expand the study to include 37 patients.

"There is no standard of care for relapsed, refractory AML, and outcomes are dismal for this patient population," Zeidner said. "Outside

of clinical trials, the conventional treatment is multi-agent, intensive cytotoxic chemotherapy."

In their study, three patients were able to proceed to stem cell transplant, which is the best chance of cure for patients with AML, and they continued to have a complete response. Five patients received "maintenance" pembrolizumab after an initial response to combination of chemotherapy and pembrolizumab to see if the drug could improve durability of the response.

The researchers paired pembrolizumab with chemotherapy because they believed the [immunotherapy drug](#) would not be effective alone. Zeidner said patients at the time of diagnosis or relapse often need treatment right away because there is "so much disease" burden, and checkpoint inhibitors drugs, such as pembrolizumab, take time to work.

If the study achieves its endpoint of 40 percent of patients in complete remission, the researchers will focus on identifying biomarkers unique to patients who respond to [pembrolizumab](#).

"We believe this drug is not going to be effective for every patient," Zeidner said. "It behooves us to find the right subgroup of patients who may ultimately have good, long-term outcomes with this [drug](#)."

Provided by UNC Lineberger Comprehensive Cancer Center

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