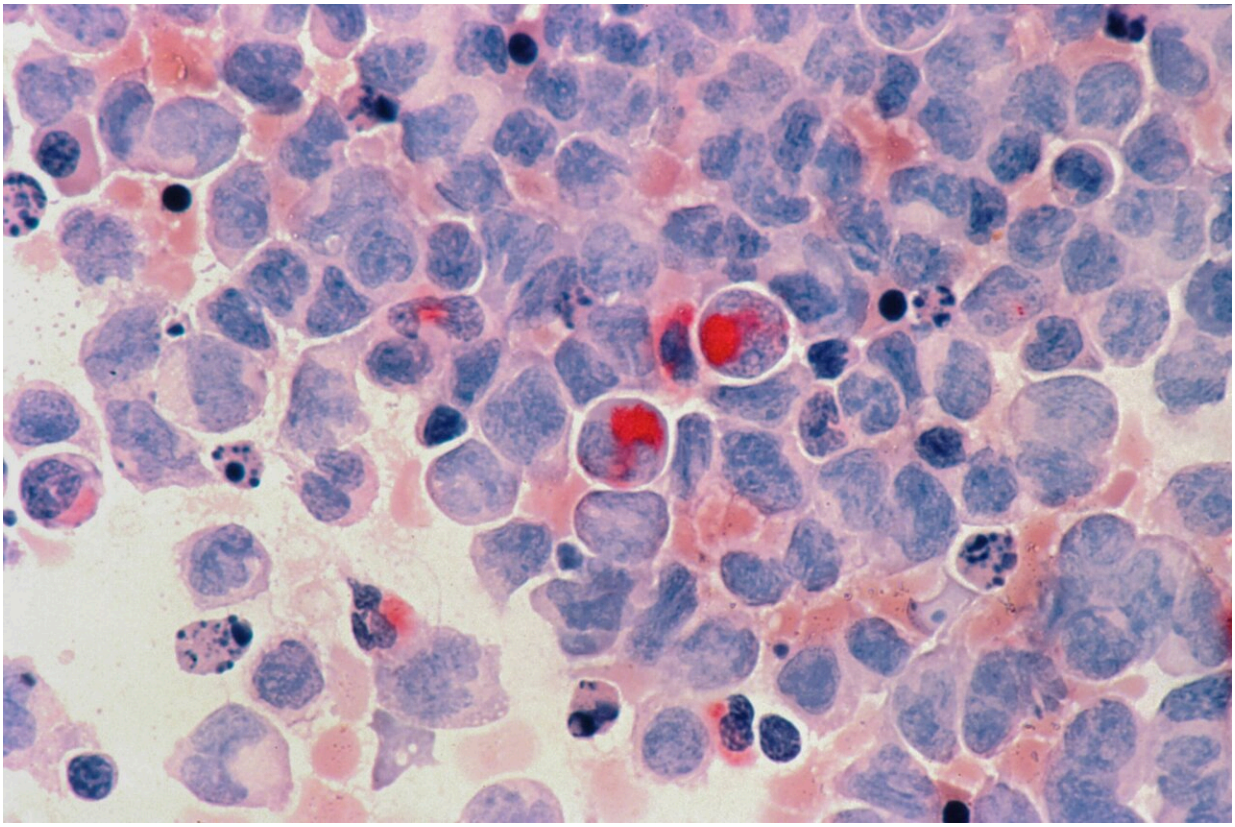
MedicalXpress

Improving treatment for hairy cell leukemia

January 13 2023, by Melissa Rohman



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Patients with relapsed or refractory hairy cell leukemia who were treated with vemurafenib experienced excellent response and relapse-free survival, according to a recent clinical trial published in *Blood*.

The results suggest that vemurafenib is an effective second-line

treatment option for these patients.

"This finding is important because it represents a targeted and relatively nontoxic and nonchemotherapeutic treatment," said Martin S. Tallman, MD, professor of Medicine in the Division of Hematology and Oncology, director of Faculty Mentorship and Career Development at the Robert H. Lurie Comprehensive Cancer Center of Northwestern University and a co-author of the study.

Hairy cell leukemia (HCL), a rare type of leukemia, manifests when bone marrow produces too many lymphocytes, a type of white blood cell. The disease is generally diagnosed in adults over the age of 50 and can be treated with the chemotherapy drug Cladribine either with or without the anti-CD20 monoclonal antibody Rituximab. Unfortunately, 30 to 40 percent of patients will still relapse.

In the case of relapsed or refractory HCL, patients are prescribed vemurafenib, a small molecular inhibitor drug. The drug, which can be administered orally, blocks BRAF V600E, a key genetic mutation in HCL cells that promotes cancer cell survival.

Previous clinical trials have shown that patients with relapsed or refractory HCL demonstrate a high initial response to the drug, but long-term outcomes have remained unknown.

In the current clinical trial, investigators measured patient outcomes of 36 individuals with relapsed or refractory HCL who were treated with vemurafenib at study sites located across the U.S., including at Northwestern Medicine hospitals.

Of these patients, the investigators found that 33 percent had a complete response to the drug and 53 percent of patients had a partial response.

A 40-month follow-up evaluation revealed that 68 percent of patients experienced relapse, with an average relapse-free survival rate of 19 months. Of the 21 patients who relapsed, 14 were re-treated with vemurafenib and of these patients, 86 percent had their white blood cell levels return to normal.

Overall, patient survival was 82 percent at four years, with a significantly shorter survival rate in patients who relapsed within one year of initial treatment. Additionally, increasing drug dosage or extending treatment duration did not improve the overall treatment response.

The investigators noted that the duration of remission was shorter with each subsequent relapse and suggest that combining vemurafenib with monoclonal antibodies may shorten treatment duration and prolong remission.

The findings suggest vemurafenib monotherapy can achieve high response rates in patients with relapsed or refractory HCL with acceptable side effects, according to Tallman.

"A randomized trial comparing the current standard-of-care for untreated patients of Cladribine plus Rituximab versus Vemurafenib plus Rituximab would be a logical next step. A new standard of care may emerge," Tallman said.

More information: Shivani Handa et al, Long-term outcomes in patients with relapsed or refractory hairy cell leukemia treated with vemurafenib monotherapy, *Blood* (2022). DOI: 10.1182/blood.2022016183

Provided by Northwestern University