

Global trial reveals life saving drug for acute myeloid leukemia

December 23 2020



Bone marrow aspirate showing acute myeloid leukemia. Several blasts have Auer rods. Credit: Wikipedia

A landmark paper published today in the *New England Journal of Medicine* describes the results from a global trial across 148 sites in 23

countries, showing a 30 percent improvement in survival in patients with acute myeloid leukemia (AML).

The Phase 3 clinical trial called QUAZAR, showed that a drug, called CC-486, significantly improved survival in older patients, over the age of 55, with the disease. AML is the most acute blood cancer in adults and its incidence increases with age, with a poor prognosis. With current treatments, the majority of older patients will die of their disease within 2 years of diagnosis.

Around 20,000 people in the United States are diagnosed with AML every year.

The global trial, led by Professor Andrew Wei from Monash University's Australian Centre for Blood Diseases and a haematologist at Alfred Health, focused on people with AML over 55 years of age, "because of an unmet need to identify new agents able to improve outcome in patients after completing chemotherapy," he said.

"After intensive chemotherapy, the risk of AML relapse is high. Many older patients are not eligible to receive a [stem cell transplant](#) and so a less toxic option to reduce disease recurrence is desirable, rather than just being monitored and waiting for the disease to come back," he said.

"Based on the results of the QUAZAR study, it is very exciting to think that, by taking a tablet that is relatively well-tolerated, we can help reduce relapse risk and improve survival."

The trial involved 472 patients, with an average age 68 years, who were either given CC-486 or a placebo.

Those receiving the drug—which has the added advantage of being a tablet that can be taken at home—had an average survival from

remission of almost 25 months compared to those who did not take the drug, whose average survival was almost 15 months.

Following Professor Wei's presentation of the results at the American Society for Hematology meeting in the United States in December 2019, the Food and Drug Administration fast-tracked approval for the use of CC-486 in the U.S in September this year.

Professor Wei says the data presented in the NEJM is likely to establish a new standard of care for [older patients](#) with AML, "because our findings show that CC-486 significantly delays recurrence of the [disease](#), thereby prolonging survival and without impacting on quality of life".

"This is a very significant advance because the drug is easy to administer and means that adults with AML don't have to spend extra time in hospital," he said.

Professor Wei added the [drug](#) is currently not approved for use in Australia.

More information: *New England Journal of Medicine* (2020). [DOI: 10.1056/NEJMoa2004444](#)

Provided by Monash University

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