New treatment of acute myeloid leukemia achieves remarkable results in a disease formerly with little hope
21 March 2019

The prognosis for older patients with acute myeloid leukemia (AML) is poor: very few achieve remission and for those that don't the option is largely palliative.

Every year almost 1000 Australians die of the disease and clinical trials into new therapies for older patients have largely failed.

A new Australian drug trial has achieved a remarkable result, clearing the bone marrow of leukaemia in almost 60% of patients.

The trial was considered so effective that the US Food and Drug Administration approved its use last November for the treatment of AML.

Kaye Oliver, 74, was the first patient in the world enrolled on this trial at the Alfred Hospital in 2015—the results of which are published today in the Journal of Clinical Oncology.

Given little hope of survival beyond a few months at diagnosis, Kaye remains well and without evidence of the cancer four years later.

Associate Professor Andrew Wei, from the Alfred Hospital and Monash University Clinical School, commenced research in this area almost two decades ago at the Walter and Eliza Hall Institute of Medical Research. He is now the lead clinician/researcher on the international trial of the cancer drug, currently combined with cytarabine to treat older adults with AML.

Taken separately these drugs achieve little, according to Associate Professor Wei. Venetoclax alone led to a 19% response rate in a US trial and cytarabine had a similar result, he said.

"But combining LDAC with venetoclax in older patients led to a 54% response rate, with half the study population surviving longer than 10 months," he added.

The trial tested 82 patients with a median age of 74 years and was conducted in Australia, Europe and the USA.

The current research is supported by another trial in older AML patients, which combined venetoclax with another drug, azacytidine and led to a 71% remission rate with an average life expectancy of almost 17 months.

Based on the early results of these two studies, the Food and Drug Administration in the US approved the use of these combination drug therapies in older people with AML on November 21 last year.

The drug combination acts on a protein prevalent in leukaemia cells called BCL-2 which controls the survival of the cells. Venetoclax acts by effectively switching off the protein and activating a self-destruct program in the cell.

Associate Professor Wei said that a randomised trial of the therapy, where patients on the therapy are compared to those who are not, has recently been completed and the results are awaited to support a submission to the Therapeutic Goods Association in Australia.

The findings are important not just because of the success of the treatment in a disease that, previously, was fatal, but because with an aging population AML is likely to become more prevalent in the future.

"AML arises due to mutations accumulating in the bone marrow over time. It also arises in patients who have previously had chemotherapy. With an
expected doubling in the number of over people over 65 in the next 30 years, the need to find more effective treatments for this disease is paramount," Associate Professor Wei said.

"AML research used to be likened to a 'clinical trial graveyard' because trials of new drugs into AML were rarely successful," Associate Professor Wei said.

"It was widely seen as an untreatable and inevitably fatal condition for older patients by most doctors. These two new trials have given real hope to patients who previously had little."

Provided by Monash University


This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.