Leukaemia Foundation says early trial results of new drug ABT-199 is good news for people with leukaemia

The Leukaemia Foundation of Queensland is encouraged by preliminary findings of a world-first Phase I trial in Melbourne of a new compound that shows great promise for people with the most common form of leukaemia.

In the ABT-199 clinical trial, people with chronic lymphocytic leukaemia* (CLL) who had an extremely poor prognosis achieved an 84% response rate and the bone marrow cancer was cleared in 23% of people.

The trial follows the work by researchers at the Peter MacCallum Cancer Centre, The Royal Melbourne Hospital and the Walter and Eliza Hall Institute. The research team discovered how to ‘switch off’ cancer cells, and some of the early laboratory development of the therapy was funded through the Leukaemia Foundation’s National Research Program in 2012.

“The findings from Phase I trials of the ABT-199 is good news for people with CLL,” said Mr Bill Petch, the Leukaemia Foundation of Queensland’s Chief Executive Officer.

“This drug means people with an incurable cancer, who undergo many periods of intensive treatment, periods of remission and with an expectation that the disease will relapse, now have hope that a treatment will be made available to end this life-long cycle,” he said.

Being a Phase I trial, a small number of people were treated, essentially to gauge the correct dose. The drug’s effectiveness will be assessed, as well as the overall side-effects, in several hundred patients in the Phase II and Phase III trials that will follow.

“Having Phase II and III trials of this drug is a great opportunity for Australians with CLL who have had previous therapy and may have no other treatment options at this time,” said Mr Petch.

“Clinical trials can give access to important therapies to people with cancer many years before they are available to them through the PBS system.

“Where possible, the Foundation encourages treating centres to offer access to clinical trials to give Australians with blood cancer access to cutting-edge, potentially life-saving and life-enhancing treatments.

“Participation contributes to the advancement of medicine and helps others who may develop the same condition in the future,” Mr Petch said.

Given the potential of this drug for people with CLL, and that it could be another five to 10 years before the drug becomes available on the PBS, the Foundation supports ongoing conversations within the Federal government about the introduction of a fast-track program for access to cancer therapies, similar to the Cancer Drugs Fund in the UK. This is an initiative we fully support.
“In Australia right now it’s so hard and takes so long to get a new drug approved, and in the meantime, people are denied access to drugs that could potentially save their lives,” Mr Petch said.

**How the therapy works**

The drug ABT-199, given as a tablet once a day, works on a protein (called Bcl-2) that prevents CLL cells from dying. Because these cells don’t die, and are still being made in the bone marrow, they begin to crowd out the blood, bone marrow and organs, such as the spleen and liver.

The protein also protects the malignant CLL cells from chemotherapy, making them resistant to this form of treatment.

ABT-199 switches off the Bcl-2 protein, allowing the CLL cells to die naturally and making them susceptible to chemotherapy, so it is more effective in killing cancer cells.

**ENDS**

*About chronic lymphocytic leukaemia*

An estimated 1000 Australians are diagnosed with chronic lymphocytic leukaemia (CLL) each year, making it the most common type of leukaemia. The risk of developing CLL increases with age and almost 80% of all new cases are diagnosed in people over the age of 60 years. CLL is rare in people under 40 and occurs more frequently in men than in women.

This slow growing leukaemia affects developing B-lymphocytes (also known as B-cells), which are specialised white blood cells. For many people, CLL remains stable for many months and years, and has little impact on their lifestyle or general health. Around 30% of people with CLL never require treatment and can survive for many years despite their diagnosis.

For others, the leukaemic cells multiply in an uncontrolled way and these abnormal cells are not able to function properly. These cells live longer than they should and accumulate in the bone marrow, blood stream, lymph nodes (glands), spleen, liver and other parts of the body. Over time, an excess number of lymphocytes crowd the bone marrow and interfere with normal blood cell production. The bone marrow then produces inadequate numbers of red cells, normal white blood cells and platelets. This leads to some people with CLL being more susceptible to anaemia, recurrent infections and bruising and bleeding easily. Circulating red blood cells and platelets also can be damaged by abnormal proteins made by the leukaemic cells.