

New drug providing hope for babies with aggressive Acute Lymphoblastic Leukaemia

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A breakthrough new drug is providing hope to tiny babies at risk of dying from an aggressive form of Acute Lymphoblastic Leukaemia and could help all cancer patients.

A team from Children's Cancer Institute in Sydney has proven a ferocious form of the blood [cancer](#) that kills half the infants who contract it, became undetectable in mice treated with [chemotherapy](#) and the new [drug](#) CBL0137.

But Children's Cancer Institute researcher Dr. Klaartje Somers says it has even greater promise because it can be used in all types of cancer including solid tumours like breast and [bowel cancer](#).

Trials underway in adults in America have found the drug to be very well tolerated.

It is hoped children in Australia whose cancer has returned will get the chance to take part in the first trial of the new drug in minors next year.

Unlike expensive new treatments like CAR T cell therapies that direct a patient's [immune system](#) towards the [cancer cells](#), this drug works by reactivating the body's P53 pathway so it kills off damaged cells. This pathway is commonly mutated in [cancer patients](#) and cancer cells suppress it so they can stay alive and keep spreading.

Dr. Somers reported in the *International Journal of Cancer* this week

that by itself the drug delayed the progression of cancer but when it was combined it with chemotherapy it worked even better.

Mice avatars were injected with blood and bone marrow samples from patients who had untreatable cancers in the study.

"When we used the molecule or the chemotherapy by themselves there was no a small response but when combined, the treatments were highly effective and deleted all the cancer cells in the mouse," Dr. Somers said.

Dr. Somers' work, under the lead of Dr. Michelle Henderson and Professor Michelle Haber, focuses on a deadly blood cancer that devastates tiny babies.

One out of ten acute leukaemia patients have a mutation in the MLL gene which means they get a very aggressive form of the disease.

Half the babies with this gene mutation will die from the disease and the aggressive chemotherapy required to treat it.

Those who survive are at risk of heart problems, [mental retardation](#), growth problems and thyroid and breast cancer as adults, caused by the aggressive chemotherapy required to kill the cancer.

Dr. Somers hopes by combining the new treatment with chemotherapy, doctors will be able to dial down the amount of chemotherapy required and reduce these side effects.

Provided by Children's Cancer Institute Australia

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