

Wilms' tumour antigen 1 Immunity via DNA fusion gene vaccination in haematological malignancies by intramuscular injection followed by intramuscular electroporation: a Phase II non-randomised clinical trial (WIN)

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Plain English summary

The WIN Phase II non-randomised clinical trial

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Plain English summary

The *Wilms' tumour antigen 1 (WT1) Immunity via deoxyribonucleic acid (DNA) (WIN)* study tested a possible vaccine treatment for patients with chronic myeloid leukaemia (CML) and acute myeloid leukaemia (AML). The treatment is directed at a molecule called WT1, which helps the survival of leukaemia cells. The vaccine also contains a small piece of genetic information from the tetanus bacterium, *Clostridium tetani*, which, linked to the gene element from WT1, is designed to boost the immune system to make white blood cells (lymphocytes) that can see and kill leukaemia cells. During the study, two types of molecules in the blood that are markers for leukaemia were measured: one is WT1 itself, the other is called *BCR-ABL* (breakpoint cluster region-Abelson murine leukaemia viral oncogene homolog 1). The vaccine was administered with a new type of injection called electroporation, designed to make the vaccine work better.

Twelve patients with CML were vaccinated. It was disappointing that the study was not completed for various reasons, the main one being slow recruitment. The levels of *BCR-ABL* were found to be reduced in one patient, whereas the levels of WT1 were reduced in another patient. The vaccine was well tolerated and there were no safety concerns. Immune response was evaluable in 10 patients. All responded to the tetanus component of the vaccine; 70% also made immune responses [CD8+ (cluster of differentiation 8)-positive T lymphocytes] to WT1. The vaccine had stimulated immune responses as we had hoped. Evaluation of the p.DOM-WT1 vaccines in AML remains clinically attractive and combination of the DNA vaccine with booster strategies is in development in the laboratory.

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