Gene therapy could fight leukemia in Down syndrome children

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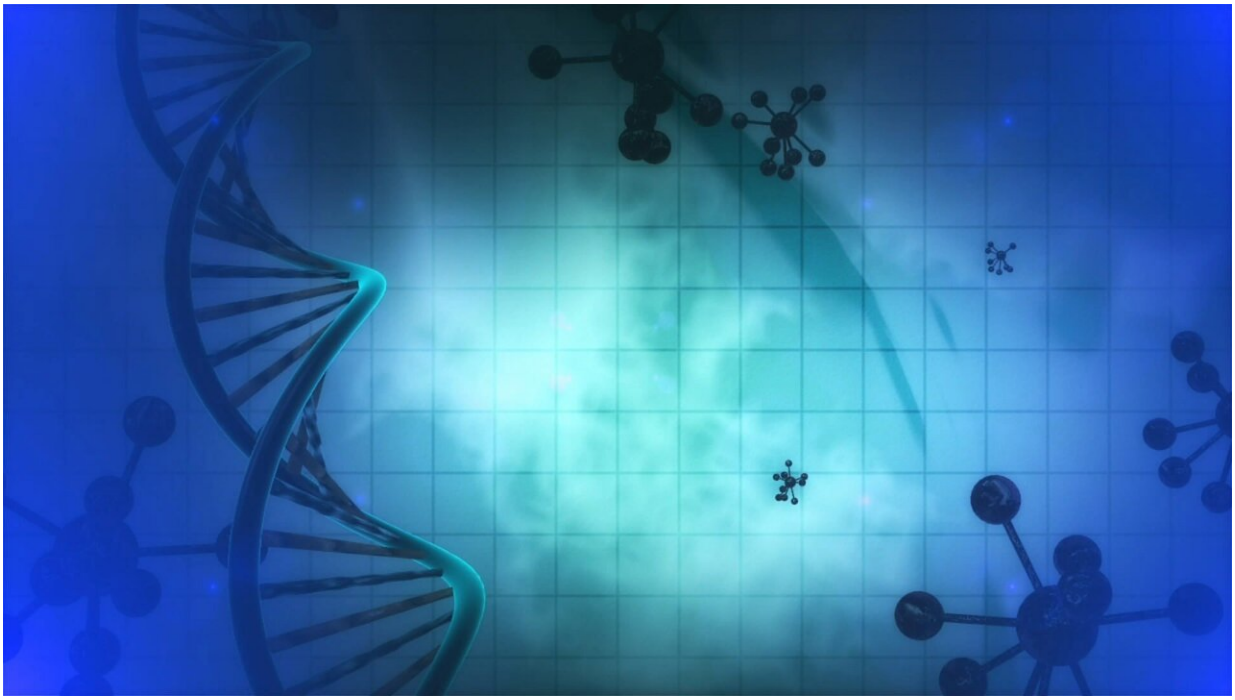

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Children with Down syndrome (DS) are 20 times more likely to develop leukemia. Not only are they more likely to develop the disease, they have higher rates of relapse, are more likely to experience negative side effects from chemotherapy and have lower survival rates.

We don't know why but genes found on chromosome 21 are thought to

play a role—of particular significance to people with DS.

Most people have 46 chromosomes in each of their cells, while people with DS have 47 thanks to an extra chromosome 21.

"The gene HMGN1 that is found on chromosome 21 is higher in all Down's syndrome patients," says post-doctoral Research Fellow, Elyse Page, who used gene editing approaches to explore the role the gene plays in leukemia development.

Previous studies in mice have shown that "switching off" HMGN1 significantly reduced leukemia and increased survival rates.

The findings suggest a possible target for new therapies.

"By knocking out this gene, we could see leukemia having a lower toll on the body, with higher survival rates and fewer side-effects from chemotherapy," Elyse says.

"We can switch off these genes with available drugs and potentially create personalized therapies for patients to decrease some of the toxic elements of chemotherapy that DS patients currently experience."

Re-purposing effective drugs that are already available to treat other diseases allows a quicker turnaround time from the lab to the clinic as their safety and side effects have already been studied.

In collaboration with national institutions, Elyse's findings will be confirmed in leukemia patients across Australia in order to establish a clinical trial for personalized therapy.

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