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Leukaemia patients near death 'saved' by edited genes

Two children are among four people in remission after artificial immune cells were infused into their blood in a trial treatment

Jonathan Leake Science Editor

Two children and two adults on the brink of death from late-stage leukaemia are in remission after infusions with genetically engineered immune cells in medical trials in London.

The "universal immune cells" had had their DNA edited so they could recognise leukaemia cells and destroy them, a task that natural cells do badly.

Professor Waseem Qasim, an immunologist at Great Ormond Street Hospital, who designed the artificial immune cells, said: "In theory, these artificial immune cells could be mass produced ready to treat patients when they are diagnosed with leukaemia."

The trials followed the 2015 case of Layla Richards, then aged one, who was dying of acute lymphoblastic leukaemia when she was given a prototype of the then untested treatment at the hospital. She has been in remission ever since.

Leukaemia is a cancer of the bone marrow which disrupts production of the white blood cells that fight off infection. It is renowned for the speed with which it develops, for targeting young people and for its growing prevalence. Each year about 10,000 people are diagnosed with leukaemia and 5,000 die from it.

The results, discussed at a conference on gene editing at the Royal Society last week, have emerged from two separate trials. One was on children aged up to 16, at Great Ormond Street, and the other on adults. Only a few patients were involved because the aim was to assess how safe the artificial cells might be. Any mainstream treatment remains some way off.

"Of the five patients in the children's trial, all went into remission," said Qasim. "One has since died from other complications while two more had their leukaemia come back and also died, but two have gone six months without disease."

Professor Paul Veys, director of the Great Ormond Street bone marrow transplant unit, who works with Qasim, said: "As they were all close to death this is a very encouraging result."

The second trial, on adults, is at King's College Hospital, in south London, with seven patients treated so far – all of them "on the brink of death", said Dr Reuben Benjamin, a consultant haematologist. Of the five that went into remission, two remain clear, a third has relapsed and the remaining two have died of separate infections.

"This is a safety trial – not a therapeutic one, so we are very encouraged to get these results so early on," said Benjamin. "The same approach could be applicable in other blood and bone cancers such as acute myeloid leukaemia, and myeloma while, in America, a related therapy licensed for treating certain types of leukaemia is coming to the market."

Layla Richards is in remission after the treatment, but was not part of the trial

The new treatment starts by extracting immune cells from the blood of a healthy donor. Such "T-cells" normally roam the body, hunting and destroying viruses or defective cells that might trigger disease.

The T-cells are then treated with molecules that splice a new gene into their DNA – reprogramming them to hunt leukaemia cells instead – and infused into a patient's blood. A second genetic modification protects the T-cell from being targeted by medications.

"Leukaemia in adults is a really nasty disease, with about a 40% survival rate," said Benjamin. "This treatment is a potential game changer. In the space of a few years gene editing has given us a possible treatment for all these diseases. We need to be cautious but also hopeful."

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