Gene therapy saves life of one-year-old with leukemia in medical first

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

For the first time ever, a person's life has been saved by gene editing.

One-year-old Layla was dying from leukaemia after all conventional treatments failed. "We didn't want to give up on our daughter, though, so we asked the doctors to try anything," her mother Lisa said in a statement released by Great Ormond Street Hospital in London, where Layla was treated.

And they did. Layla's doctors got permission to use an experimental form of gene therapy using genetically engineered immune cells from a donor. Within a month these cells had killed off all the cancerous cells in her bone marrow.

It is too soon to say she is cured, the team stressed at a press conference in London on 5 November. That will only become clear after a year or two. So far, though, she is doing well and there is no sign of the cancer returning. Other patients are already receiving the same treatment.

The basic idea is to remove immune cells from a patient's body, genetically engineer them to attack cancerous cells and place them back in the body. Several human trials are already underway around world. Some trials involve adding a gene for a receptor called CAR19, which sits on the outside of the T-cells. This programs the T-cells to seek out and kill any cells with a protein called CD19 on their surface – which is found on the cells that cause acute lymphoblastic leukaemia.

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