Case study: Non-heritable genome editing for medical treatments

Purpose: Using genome editing to provide new treatments for diseases, such as leukaemia, that have resisted other forms of treatment. These treatments involve making changes to somatic cells, which include all the cells in the body that are not involved in reproduction. As a result, changes made to these cells cannot be inherited by any children of the person receiving the treatment.

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The global challenge

Leukaemia is the most common childhood cancer, accounting for 30% of all cancers diagnosed in children under 15 years of age in industrialised countries. Five year survival rates (the proportion of children diagnosed with leukaemia who are alive five years after diagnosis and treatment) range from 52.4% in Colombia to 91.6% in Germany.

Established treatments include chemotherapy and bone marrow transplant. For patients in which these treatments have been tried unsuccessfully, there is an emerging treatment that involves extracting a patient's own T-cells (a type of immune cell that targets infected cells within the body, including cancers), genetically engineering them to be more effective at targeting leukaemia and then returning them into the patient.

UK facts & figures

- There are over 500 new cases of leukaemia diagnosed every year in children aged under 15
- The five year survival rate for these children is over 85%
- Leukaemia is the most common cancer in children under the age of five.

A genetic technologies example

In 2015 doctors at Great Ormond Street Hospital pioneered a new treatment for leukaemia in an 11 month old girl, Layla Richards, whose cancer had not responded to the conventional treatments. Layla also did not have enough of her own T-cells to use the emergent treatment of modifying her own cells. So in baby Layla's case doctors used T-cells from a donor. They also pioneered a genome editing technique to adapt these cells so they not only targeted the leukaemia but also would not be rejected by her immune system or affected by the chemotherapy drugs.

The treatment effectively targeted Layla's leukaemia and two years later it has not returned. The same technique has since been used to successfully treat another infant, although further successful trials would be required before the treatment is made more widely available. Both children will be monitored over the long term to check for complications from the treatment or the return of the cancer.

Arguments in favour of developing somatic genome editing treatments

- Genome editing is opening up new treatment opportunities for otherwise intractable diseases, including certain types of cancer and HIV, as well as genetic diseases such as cystic fibrosis
- Editing donor cells opens up the possibility for cheaper, readily available genetic therapies for leukaemia.

Arguments against developing somatic genome editing treatments

- Long term efficacy and possible side-effects of these treatments are unknown
- Compared with some conventional treatments, genome editing is currently very expensive
- Some somatic treatments might also have the potential for enhancing basic human characteristics such as height or strength.

The alternatives

In this specific case study, the somatic genome editing treatment was used where all available alternatives had already been tried.