

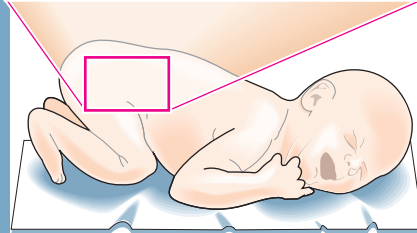
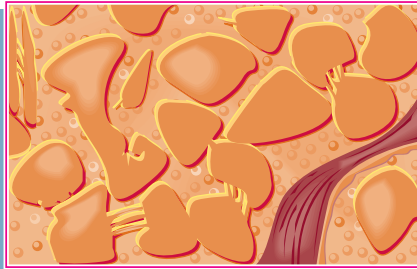
GENE THERAPY

Carly's last chance for life

Eight-month old Carly Todd is the first patient to benefit from a technique developed by British and Dutch scientists which could prove a breakthrough in the treatment of hereditary diseases. Approval had first to be secured from the Clothier committee, appointed to examine the ethics of gene therapy

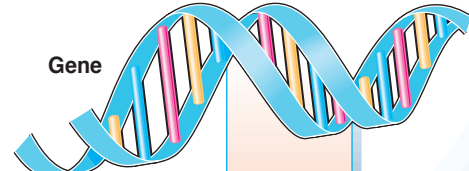
THE PROBLEM

Carly suffers from Severe Combined Immunodeficiency – or total allergy syndrome. She lacks the ADA gene (Adenosine Deaminase) without which toxic substances accumulate in the bloodstream and damage the immune system. The missing gene is a chromosome segment containing the genetic instructions to make ADA. **Sufferers usually die in early childhood**



THE TREATMENT

1 Thursday, March 18: Bone marrow is taken from Carly's pelvic bone by Dr Gareth Morgan at London's Great Ormond Street Hospital. The bone marrow is flown to the Netherlands



2 Carly's missing gene is isolated and cut out of a healthy donor cell by Professor Tinco Valerio at the TNO Research Institute in Delft

Gene spliced into virus

Virus

4 The virus carrying the missing gene enters one of Carly's stem cells and becomes part of the nucleus. Stem cells in the bone marrow produce blood cells

5 The genetically altered stem cell is reproduced in an incubator, creating millions of cells each containing the missing gene

Cells multiply

3 The virus is rendered harmless by removing the genetic code which enables it to reproduce

Virus is allowed to infect bone marrow cell

6 Monday, March 22: The stem cells are injected into Carly's bloodstream back at Great Ormond Street Hospital

7 The stem cells migrate naturally to Carly's bone marrow where they begin to produce healthy blood cells complete with the ADA gene – capable of fighting infection

