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Gene therapy - Horizon “Trial and Error”

October 18th, 2006

The excitement about gene therapy received a serious blow in September 1999 with the death of Jesse Gelsinger. At the time 18 year old Jesse was a participant in a clinical trial for gene therapy to overcome a genetic condition Ornithine Transcarbamylase (OTC) deficiency. This documentary “Trial and Error” from the BBC’s flagship Horizon series tells the story of that trial and what went wrong. Although the tone is sometimes unduly sensationalist, it is an excellent introduction to gene therapy. The story is complicated by serious flaws in the conduct of the clinical trial, which can also make this a good vehicle for discussing appropriate procedures in biomedical research.

This episode is from the 2003 season of Horizon. It is frequently repeated on digital TV, particularly the UKTV Documentary channel. It is also available as an off-air recording from the BUFVC (TRILT identifier 001D70CE). Watching the whole episode is time well spent. However, a four and a half minute clip starting at 4:40 with the voiceover “A medical revolution had begun...” and continuing through interviews with French Anderson and Dusty Miller explains the principles of using viral vectors for delivering genes into cells. [A transcript of the programme](#) is available on the BBC website.

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[What are BUFVC and TRILT?](#)
[What's BioethicsBytes all about?](#)

CATEGORIES

[Animal experimentation](#) (1)
[Bioethics Briefing](#) (1)
[Book](#) (3)
[Clinical trials](#) (1)
[Euthanasia](#) (1)
[Film](#) (2)
[Gene therapy](#) (1)
[Human cloning](#) (3)
[Human tissue usage](#) (3)
[Play](#) (1)
[Podcast](#) (3)
[Research Ethics](#) (1)
[Stem Cells](#) (7)
[Streamed video](#) (1)

Bioethics News Stories: a top ten

- E-mail survey, October 2006
- n=23 replies
- Rough and ready, but some interesting observations
- 18 stories got 2 votes and >50 got 1 vote

6=

Foetal material used
in transplants

Diane Blood's battle to use dead husband's sperm to have baby

No stopping my son, says Diane Blood



Diane Blood: "All my thoughts are for Liam"

Widow Diane Blood, who fought a legal battle for permission to use her dead husband's sperm, says there is no stopping her baby son.

6=

Last Updated: Tuesday, 24 January 2006, 13:08 GMT

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Clinic assists doctor's suicide

A retired doctor has taken her own life with the help of doctors at a controversial Swiss clinic.

Dr Anne Turner, from Bath, had a progressive and incurable degenerative disease called supranuclear palsy.



[▶ VIDEO](#) [Dr Turner interview](#)

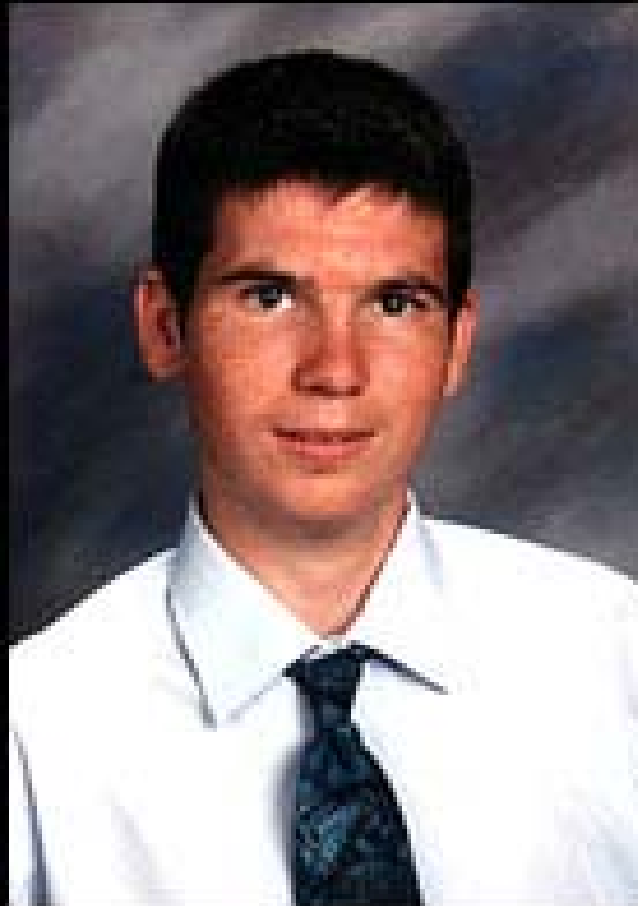
She travelled to the Dignitas clinic in Zurich on Tuesday, where doctors gave her drugs with which to end her life.

Anne Turner PAS in
Switzerland

6=

6=

Jesse Gelsinger
dies in gene
therapy trial



Second cancer case halts gene-therapy trials

Erika Check, Washington

The world of gene therapy was shaken last year when a child treated in a French trial developed leukaemia. Researchers had pinned their hopes on this being an unfortunate one-off. Now those hopes have been dashed with the emergence of a second, almost identical case that could jeopardize the future of gene therapy.

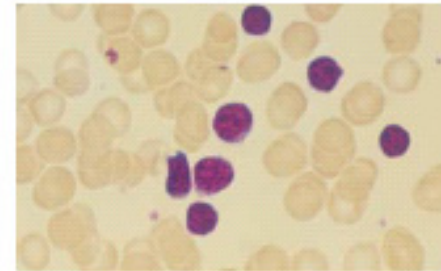
The latest case centres on a three-year-old boy treated in a gene-therapy trial led by Alain Fischer at the Necker Hospital for Sick Children in Paris. Just under three years ago, the child was cured of severe combined immunodeficiency disease (SCID), a condition that disrupts the development of the immune system. But researchers revealed last week that the boy was diagnosed with leukaemia just days before Christmas.

The news last August that a child in Fischer's trial had developed cancer left nations divided over their regulatory response (see

gene therapy's only true success. Fisher has so far cured nine boys — including the two who now have leukaemia — out of 11 patients. And when the first child was diagnosed with cancer, some argued that it was an isolated event (see *Nature* 420, 595; 2002).

But Christof von Kalle of the Cincinnati Children's Hospital says analysis of the two boys' cells shows that the same molecular events probably caused the cancers. In both boys, the retroviral vector used to deliver the corrective gene has integrated itself into a stretch of DNA in or near a gene called *LMO2*, which can cause childhood leukaemias. Scientists are now satisfied that the disease behaves enough like leukaemia to describe it as such.

Kalle estimates that the vector is likely to insert itself near to *LMO2* in between 1-in-50,000 and 1-in-100,000 cells. And because each child in the trial receives a dose of about a million corrected cells, each patient could carry at least one cell with the vector near



Blood cancer: leukaemia has been detected in two patients who have received gene therapy.

to examine data from human and animal trials of retroviral vectors. And the National Institutes of Health's Recombinant DNA Advisory Committee (RAC) postponed its planned meeting on 17 January, but will convene shortly to discuss these issues.

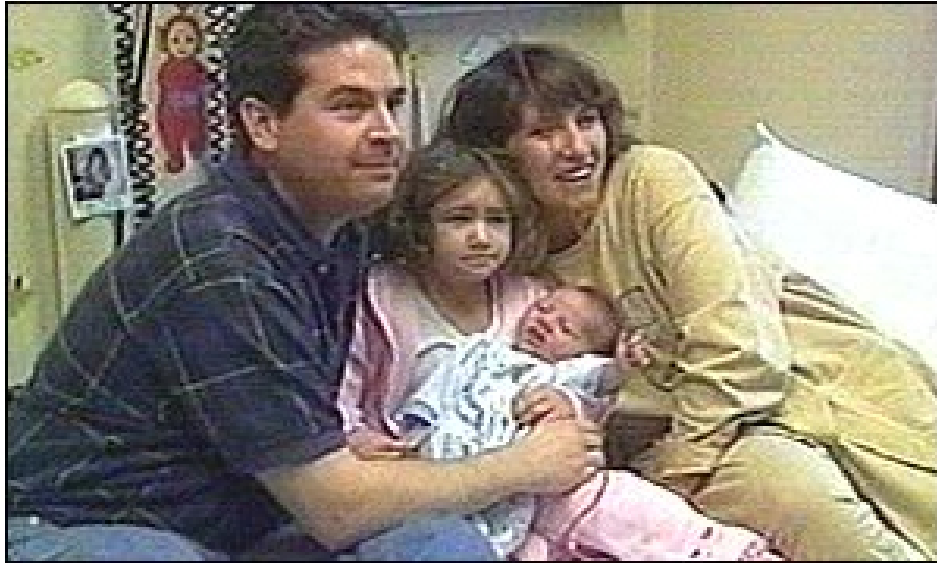
At their last meetings, both the FDA committee and the RAC recommended that gene therapy in SCID could proceed with appro-

6 =

Leukaemia after gene therapy for X-SCID

Wednesday, 4 October, 2000, 07:37 GMT 08:37 UK

Baby created to save older sister



The Nash family have created medical history

A test-tube baby has been selected by doctors using controversial genetic screening to save the life of its older sister.

6=

Pre-implantation Genetic Diagnosis to treat Molly Nash

Tony Bland in PVS
allowed to die



3=

Wednesday, 6 September, 2000, 16:09 GMT 17:09 UK

Experts back twins' separation



The girls were born at St Mary's Hospital, Manchester
Medical experts have supported proposals to separate a pair of Siamese twins at the centre of a court battle.

3 =

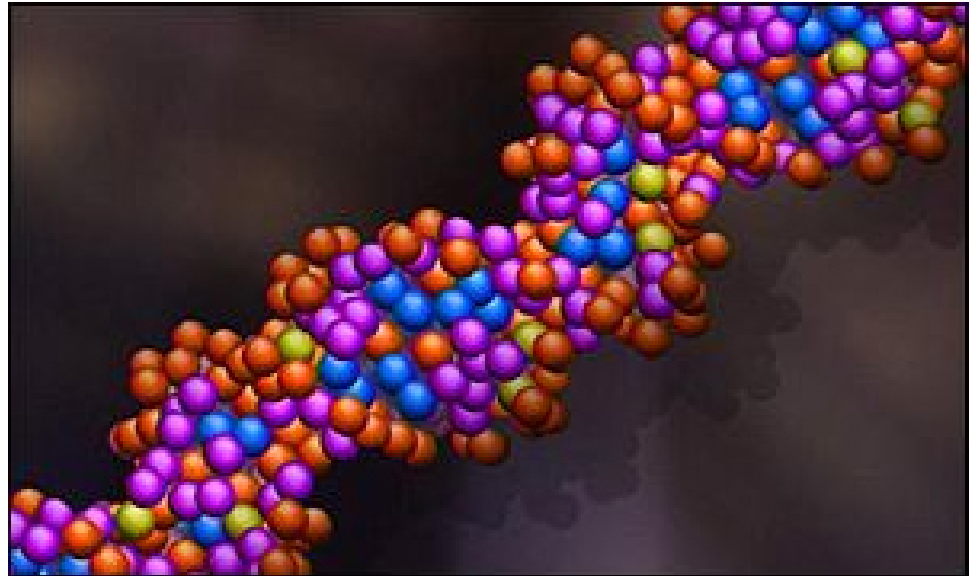
Jodie and Mary conjoined
twins separated

'Completion' of the human genome project

3 =

Monday, 26 June, 2000, 18:41 GMT 19:41 UK

Scientists crack human code



The "code of life" is hidden in our DNA

The US president and the UK prime minister have hailed the rough draft of the entire human genetic code as "the most wondrous map ever produced by human kind".

Hwang Woo-Suk fakes data

Last Updated: Friday, 23 December 2005, 08:29 GMT

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S Korea cloning research was fake

Research by South Korea's top human cloning scientist - hailed as a breakthrough earlier this year - was fabricated, colleagues have concluded.



Dr Hwang has been hailed as a hero in South Korea

A Seoul National University panel said the research by world-renowned Hwang Woo-suk was "intentionally fabricated", and he would be disciplined.

Dr Hwang said he would resign, but he did not admit his research was faked.

2

1



Cloning of Dolly the Sheep

No GM stories?

Yes, but spread over varied stories, e.g. 2 votes each for:

- Pusztai's potatoes
- Golden rice
- GM tomato paste
- GM pollen and monarch butterfly
- Soyabean contamination