Somatic Gene Therapy: History of Gene Therapy

US275 Scientific Ethics
John R. Hoffman
Arcadia University

Gene therapy studies are reviewed at an institution before seeking approval as a Clinical Trial.

- Institutional Review Board (IRB)
- Institutional Biosafety Committee (IBC)

Institutional Biosafety Committee

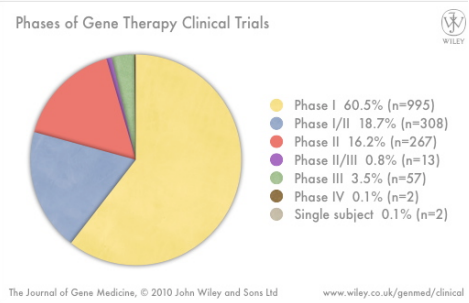
Gene therapy clinical trials are evaluated at multiple levels.

- NIH guidelines for human gene manipulation and therapy
- NIH Recombinant DNA Advisory Committee
  - reviews clinical plan for medical, ethical, and/or safety issues
- FDA
  - oversight of studies by U.S. researchers

http://scienceroll.com/2008/11/15/personalized-medicine-report-of-us-presidents-advisory-committee/

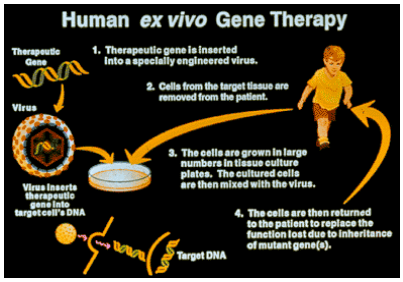
The FDA has not approved any human gene therapy treatments outside of clinical trials.

- clinical trials began in 1990
- over 1600 trials conducted



The first gene therapy clinical trial began in 1990.

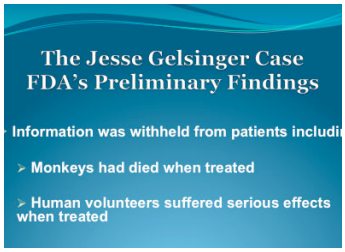
- immune system disease
  - Adenosine deaminase (ADA) deficiency
- patients white blood cells collected
  - normal gene added to cells
  - cells returned to body.



http://history.nih.gov/exhibits/genetics/sect4.htm

A patient in an early gene therapy clinical trial died in 1999.

- Clinical trial for treatment of liver disease
  - ornithine transcarboxylase deficiency (OTCD)
- patient died of multiple organ failure
  - thought to be triggered by severe immune response to adenovirus vector
  - approved protocol was not followed resulting in increased risks



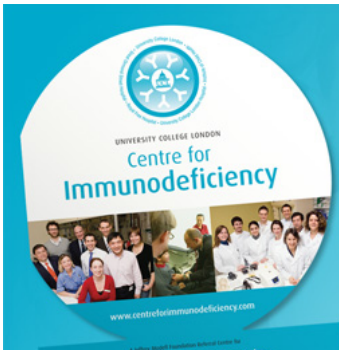
In 2003, the FDA halted all gene therapy trials using retroviral vectors in blood stem cells.

- Study had successfully treated "bubble baby syndrome"
  - X-linked severe combined immunodeficiency disease
- several patents developed leukemia-like conditions
- FDA eventually eased the ban

http://www.geneticsandsociety.org/article.php?id=3840

There are currently two uses of gene therapy in animals.

- Novartis Animal Health
  - DNA vaccine against Infectious Salmon Anaemia
- VGX Animal Health
  - growth hormone releasing hormone therapy for farmed pigs.



The most successful human gene therapy so far is for treatment of severe immune deficiency disease.

- ADA-SCID
  - bubble baby syndrome
  - Centre for Immunodeficiency in London

Assignment

Somatic Gene Therapy: a "cure"?

In somatic gene therapy, a new or replacement gene is inserted into an adult's body cells. The patient is able to make an informed decision about the risks and benefits of the treatment, and there is no risk of passing the altered genome to their children.

In your discussion group:
Discuss types of genetic conditions should be treated with somatic gene therapy. Consider different types of situations including life-threatening conditions, unpleasant conditions, or aesthetic/cosmetic conditions.

Readings

Sade RM, Khushf G. Gene therapy: ethical and social issues. J So Carolina Med Assoc 1998;94(9):406-410
http://academicdepartments.musc.edu/humanvalues/pdf/gene-therapy.pdf

Gene Therapy. Genetics Home Reference.
http://ghr.nlm.nih.gov/handbook/therapy

Reading in the Garden
Nikolai Petrovich Bogdanov-Belsky, 1915

Any Questions?

Email me at:
hoffmanj@arcadia.edu

http://www.yippitbullkennels.com/images/animated-question-mark.gif