

# Regulation of gene therapies

Bioengineering 100

Fall 2016

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# What is gene therapy?

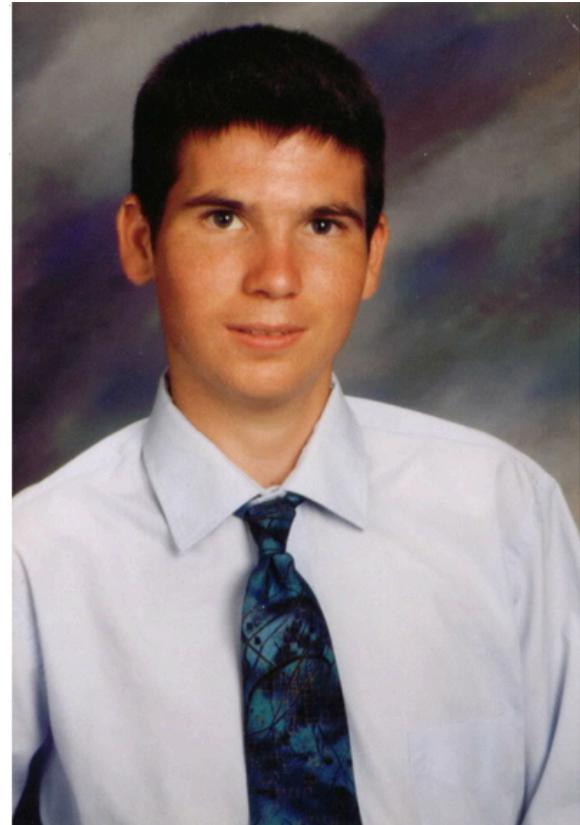
- Transfer of genetic constructs to humans
- Delivery of constructs are plasmids of viral/bacterial vectors

# Brief Timeline

- 1972: First intentional recombinant DNA molecules published
- 1974: Formation of NIH Recombinant DNA Advisory Committee (RAC)
- 1976: NIH guidelines for research involving recombinant or synthetic nucleic acid molecules (NIH Guidelines)
- 1984: FDA begins regulating gene therapy products
- 1997: FDA sole approval agency with RAC advisory role; IRB, IBC to implement on local level
- 1999: Jesse Gelsinger dies in an early gene therapy trial
- 2000: FDA strengthens clinical trial protections for participants
- 2004: NIH launches GeMCRIS, publicly accessible web database with information about gene transfer clinical trials
- 2013: NIH proposes revision to reduce oversight for low-risk protocols with existing IBC approval for prior safety trials

# Jesse Gelsinger

- First known death from a gene therapy clinical trial
- 18 years old
- Had OTC, rare metabolic disease, body cannot break down ammonia byproducts from proteins
- Treatment is low protein diet with drugs (32 pills/day)



**"What's the worst that can happen to me?" ... "I die, and it's for the babies."**

Jesse Gelsinger before he left for  
the hospital in Pennsylvania

# RAC

- Panel of experts (up to 21) including scientists, clinicians, public health experts, bioethicists, public
- Select gene therapy clinical trials protocols are reviewed at quarterly public meetings
- Meetings are webcast and detailed transcripts published
- Purpose:
  - Strengthen clinical trial design and safety
  - Provide a transparent forum for dissemination of current gene therapy research
  - Inform other regulatory agencies (FDA, OHRP, IRB, IBC etc...)

# First CRISPR clinical trial gets green light from US panel

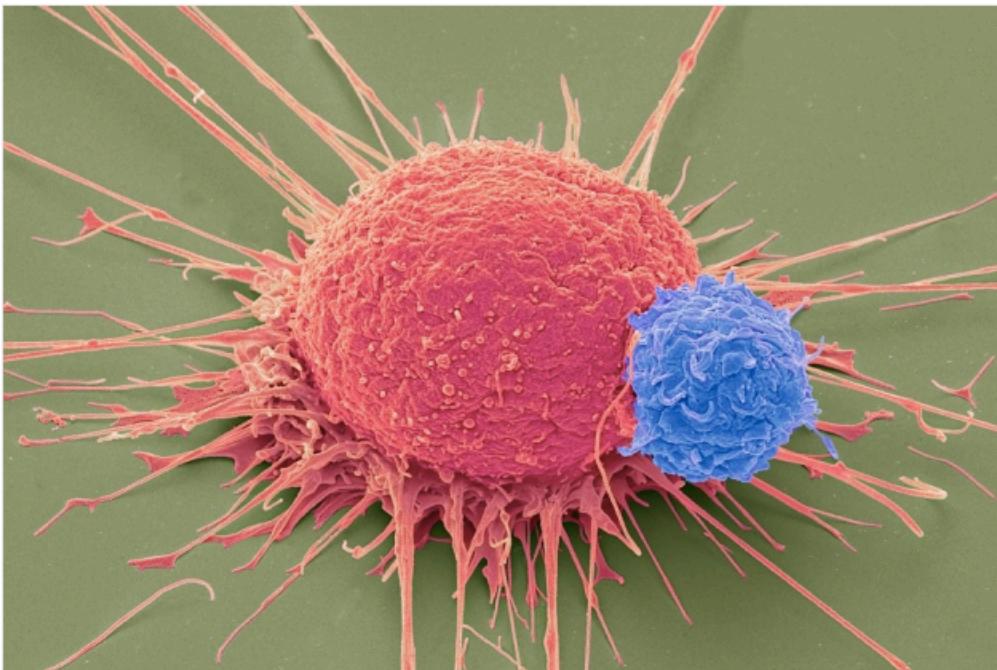
The technique's first test in people could begin as early as the end of the year.

Sara Reardon

22 June 2016



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STEVE GSCHMEISSNER/SPL

# Zika News

NATURE | LETTER [Accelerated Article Preview](#)

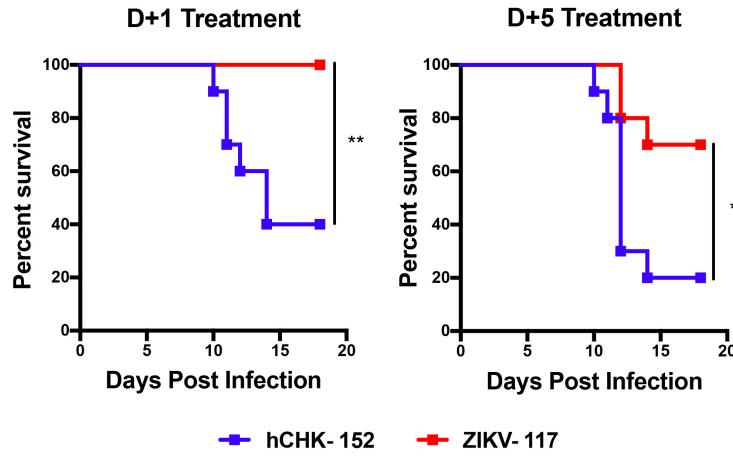
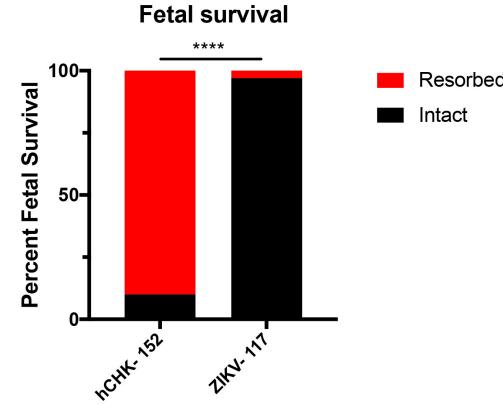
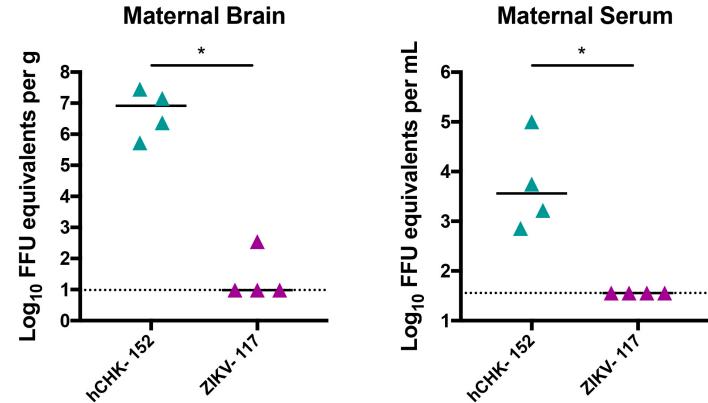
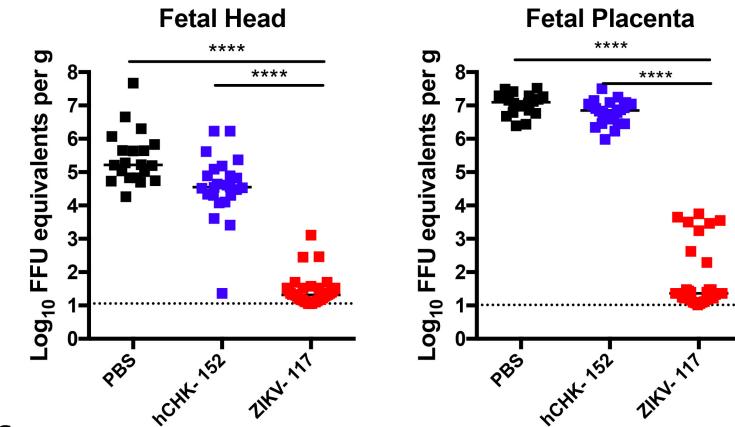
# Neutralizing human antibodies prevent Zika virus replication and fetal disease in mice

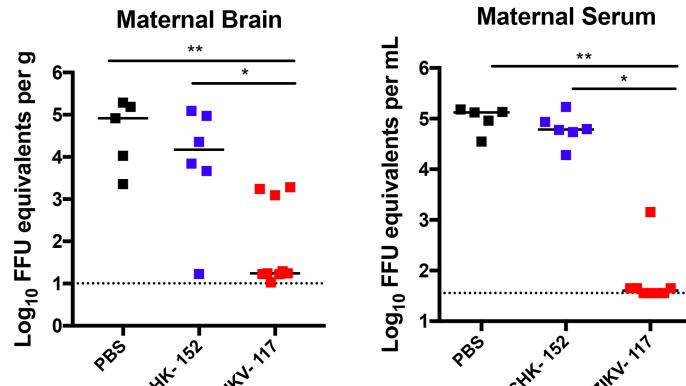
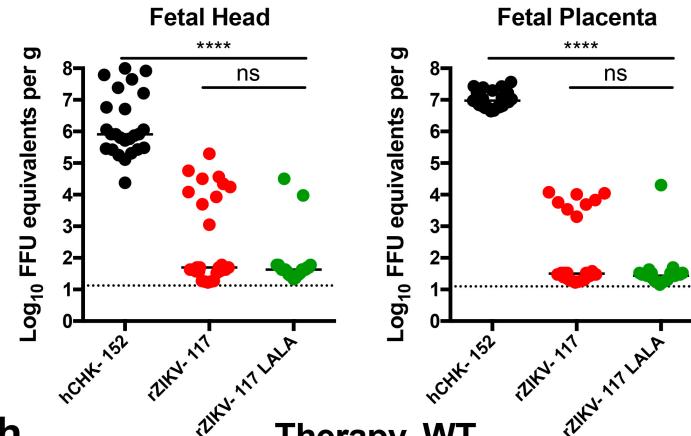
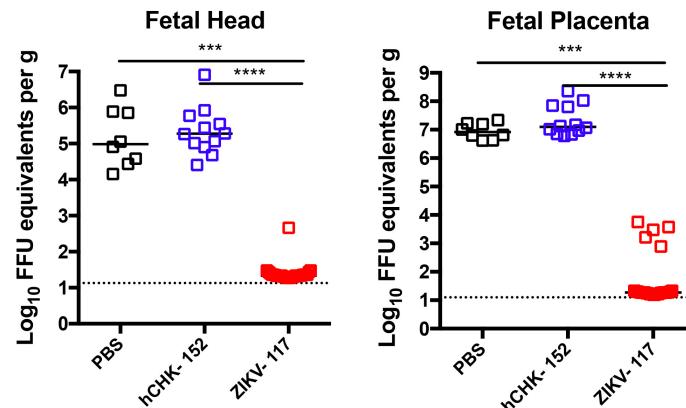
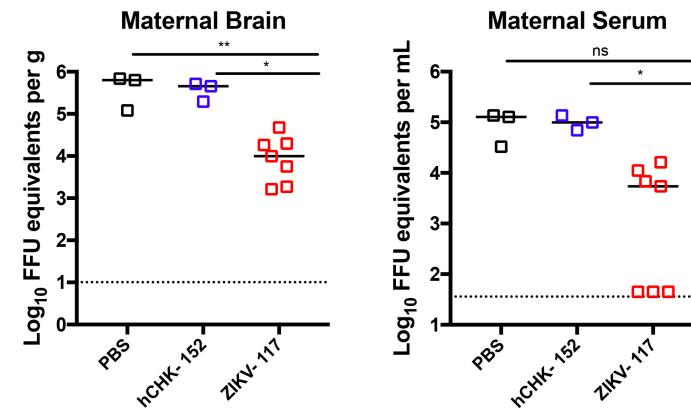
Gopal Sapparapu, Estefania Fernandez, Nurgun Kose, Bin Cao, Julie M. Fox, Robin G. Bombardi, Haiyan Zhao, Christopher A. Nelson, Aubrey L. Bryan, Trevor Barnes, Edgar Davidson, Indira U. Mysorekar, Daved H. Fremont, Benjamin J. Doranz, Michael S. Diamond & James E. Crowe

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**a** **Therapy, ♂****b** **Prophylaxis, *Ifnar*<sup>-/-</sup>****c** **Prophylaxis, *Ifnar*<sup>-/-</sup>****d** **Prophylaxis, WT**

**e Prophylaxis, WT****f Prophylaxis, WT****g Therapy, WT****h Therapy, WT**

# Election Day!!

# Have you voted today?



A close-up photograph of a caterpillar, likely a larva of the Malacosoma tigris species, commonly known as the Eastern Tent Caterpillar. The caterpillar is a vibrant yellow color with a dense, fuzzy texture. It is shown crawling along a light-colored, textured tree branch. The background is blurred green foliage, suggesting a forest environment.

“Trumpapillar”