

# **Gene Therapy: Looking to the Future**

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# Overview

- **NIH's role in the science and oversight of gene therapy**
- **Why you are here**
- **Your specific charge**



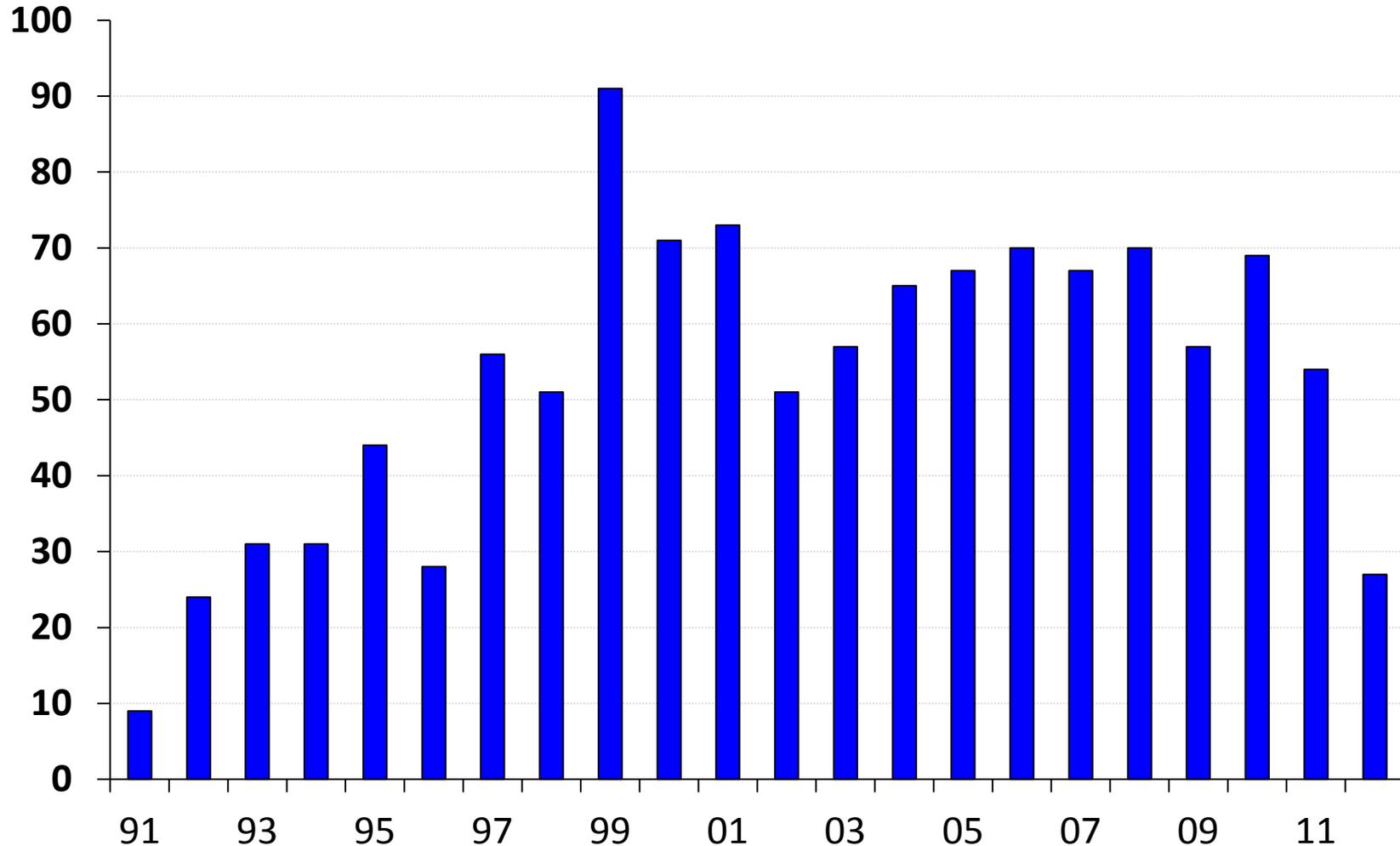


NATIONAL INSTITUTES OF HEALTH

James A. Shannon  
Director

# Number of Protocols Submitted

Total = 1, 207 (As of March 2013)



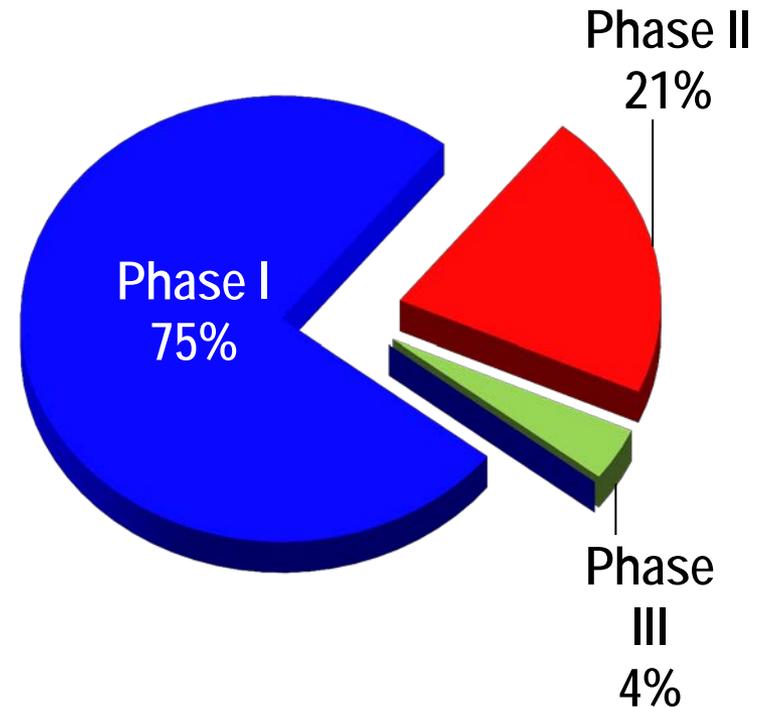
# Human Gene Transfer Trials By Phase

**1990 - 1992**



**N=35 protocols**

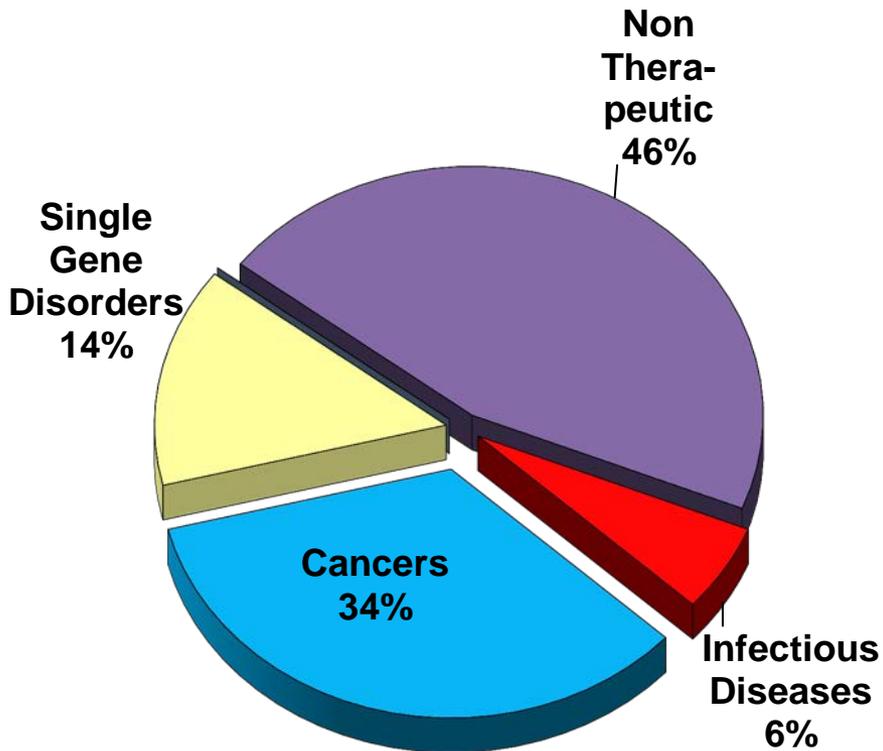
**2010 - 2012**



**N=181 protocols**

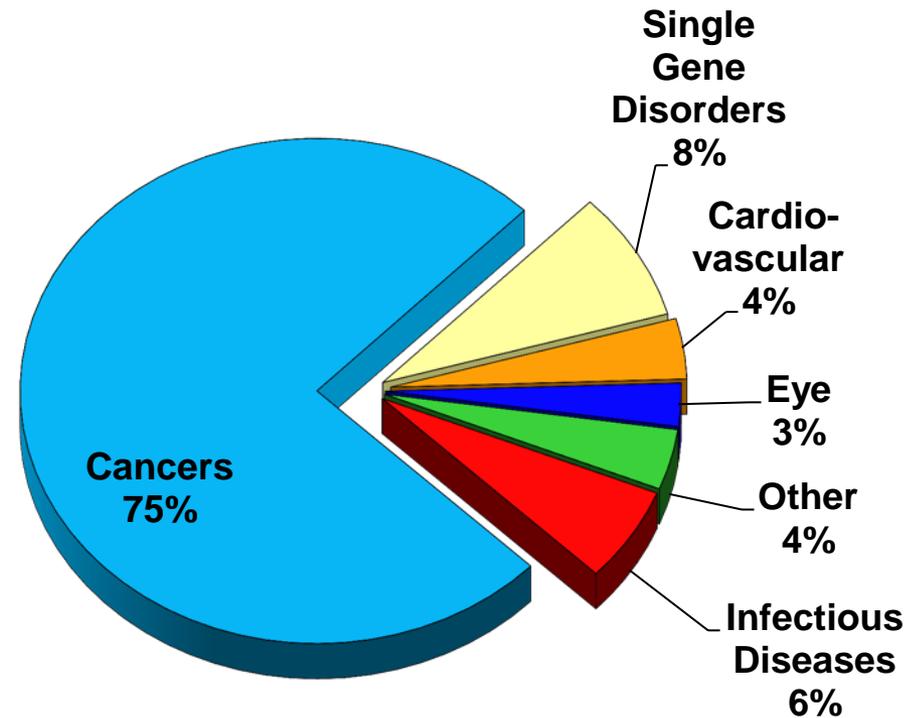
# Human Gene Therapy Trials By Clinical Indication

**1990-1992**



**N=35 protocols**

**2010 - 2012**



**N=181 protocols**

# NIH Recombinant DNA Advisory Committee (RAC)





## Support

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Welcome to the NIH Genetic Modification Clinical Research Information System (GeMCRIS®). GeMCRIS is a comprehensive information resource and analytical tool for scientists, research participants, institutional oversight committees, sponsors, federal officials, and others with an interest in human gene transfer research. GeMCRIS allows users to access an array of information about human gene transfer trials registered with the NIH, including medical conditions under study, institutions where trials are being conducted, investigators carrying out these trials, gene products being used, route of gene product delivery, and summaries of study protocols.

To facilitate access to this information, GeMCRIS offers a number of preformatted reports. You can also create your own query tailored to your particular information needs. To get started, use the "Search" menu item above, or click the "Frequently Asked Questions" link on the left to learn more about using the system.

We are seeking comments on GeMCRIS's utility and ease of use. Please take a moment to respond to the questions on the form provided through the "Feedback" link on this page. Your input is critical to ensuring that the system meets the needs of all its diverse users.



## Related Information

- ▶ [About The RAC](#)
- ▶ [NIH Guidelines](#)
- ▶ [Documents \(With Quarterly Reports\)](#)
- ▶ [Link To NGVL](#)



# Scientific, Safety, and Policy Symposia

- 2012**      **Gene Transfer and Rare Diseases**
- 2011**      **RNA Oligonucleotides: Emerging Clinical Applications**
- 2010**      **Sham Neurosurgical Procedures in Clinical Trials for Neurodegenerative Diseases**
- 2010**      **Retroviral and Lentiviral Vectors for Long-Term Gene Correction**
- 2010**      **Gene-Modified T Cells: Challenges in Clinical Trial Design**
- 2007**      **Immune Responses to AAV Vectors**
- 2005**      **Gene Transfer for X-SCID**

# Scientific, Safety, and Policy Symposia

- 2004**      **Safety Considerations in Recombinant DNA Research with Pathogenic Viruses**
- 2001**      **IBCs in a Changing Research Landscape**
- 2001**      **Adeno-Associated Virus in Gene Transfer Clinical Trials**
- 2000**      **Cardiovascular Gene Transfer Clinical Research**
- 1999**      **Prenatal Gene Transfer: Scientific, Medical and Ethical Issues**
- 1998**      **Lentiviral Vectors for Gene Delivery**

A green rectangular road sign with rounded corners and a white reflective border. The sign is mounted on two wooden posts and features the text "The Future" in a large, white, sans-serif font. The background is a clear blue sky with scattered white clouds.

The Future

# Your Charge

- **Express your individual views about:**
  1. **The most important scientific opportunities on the horizon, and**
  2. **Challenges to realizing those opportunities-- whether it is:**
    - **Knowledge gaps**
    - **Policy issues**
    - **Ethics concerns**
    - **Technology needs or other necessary resources**