Introduction to the NIH Office of Biotechnology Activities and the Resources OBA Offers Investigators

NIH Mission

- **Discover new scientific knowledge that will improve human health**
- **NIH funds, conducts, and oversees biomedical research**
  - **50,000 + extramural scientists**
  - **2,000 + research institutions**
  - **5,000 + intramural scientists**
  - **27 Institutes and Centers**

NIH Stewardship Responsibilities

- **Invest wisely taxpayer dollars entrusted to it for the support and conduct of biomedical research**
- **Communicate and apply the knowledge gained from research**
  - **Improve the design and conduct of ongoing and future studies**
  - **Efficiently advance development of new treatments and cures**
  - **Optimize patient safety**

NIH Office of Biotechnology Activities

6705 Rockledge Drive, Suite 750

NIH Office of Biotechnology Activities

- **Within the Office of Science Policy, Office of the Director, NIH**
- **Five programs:**
  - **Recombinant DNA (RAC) and Biosafety**
  - **Genetics (SACGHS)**
  - **Biosecurity (NSABB)**
  - **Clinical Research Policy (CRpac)**
  - **Outreach and Education**

Recombinant DNA Program

- **Oversee recombinant DNA research, including human gene transfer**
- **Manage the Recombinant DNA Advisory Committee (RAC)**
- **Administer the *NIH Guidelines for Research Involving Recombinant DNA Molecules***
- **Partner with Institutional Biosafety Committees in the oversight of recombinant DNA research**

Recombinant DNA Program

- **Disseminate information on technical and policy matters concerning recombinant DNA research**
  - **RAC recommendations on clinical protocols**
  - **Interpretations of the *NIH Guidelines***
  - **Scientific symposia and policy conferences**
- **Develop and contribute to public policy on recombinant DNA research**
  - **Interagency oversight of biotechnology**

Investigator Training and Education

- **Conducting training programs for investigators is a responsibility of**
  - **NIH OBA**
  - **Institutions**
- **Education and training is essential for the:**
  - **Safe conduct of research**
  - **Compliance with grant requirements**
- **Training is a priority concern:**
  - **OBA site visits to institutions reveal that investigator awareness of requirements for research involving recombinant DNA is low**
- **OBA has valuable resources to offer**

NIH OBA Training, Education, and Information Resources

- **Resources on OBA's web site**
  - ***NIH Guidelines*** **and** ***Federal Register*** **notices**
  - **"Latest news" items on meetings, policy guidance, resources, compliance notices, etc.**
  - **FAQs**
  - **Training materials: slide presentations and video of professional development workshops**
- **Training courses and presentations at key professional and scientific meetings**
- **Scientific symposia and policy conferences**

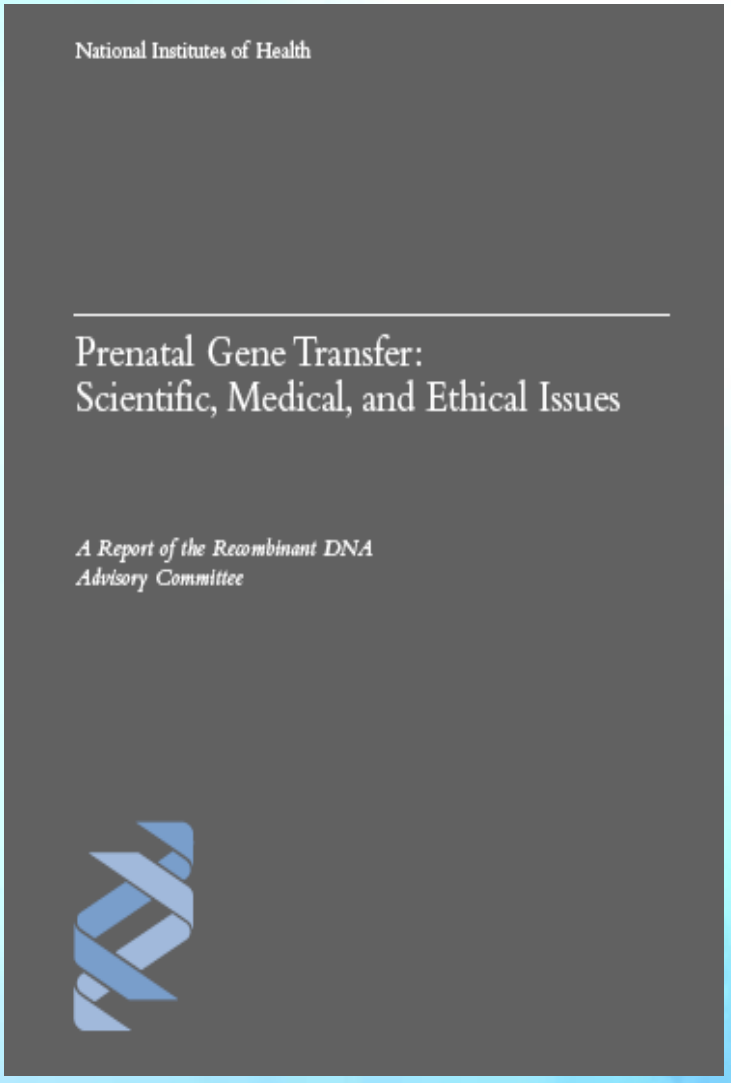
NIH Gene Transfer Policy Conferences

1997 Human Gene Therapy: Beyond Life Threatening Disease

1998 Lentiviral Vectors for Gene Delivery

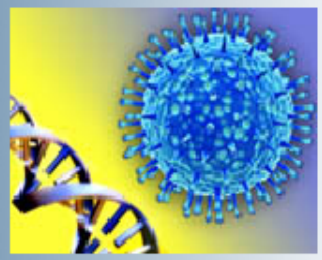
1999 Prenatal Gene Transfer: Scientific, Medical and Ethical Issues

2001 IBCs in A Changing Research Landscape



Gene Transfer Scientific and Safety Symposia

- 1999 Adenoviral Vector Safety and Toxicity
- 2000 Internally Deleted, Helper Dependent ("gutless") Adenoviral Vectors
- 2000 Cardiovascular Gene Transfer Clinical Research
- 2001 Adeno-Associated Virus in Gene Transfer Clinical Trials
- 2004 Safety Considerations in Recombinant DNA Research with Pathogenic Viruses
- 2005 Gene Transfer for X-SCID



Recombinant DNA and Gene Transfer

Office of Biotechnology Activities

Safety Considerations in Recombinant DNA Research with Pathogenic Viruses

On September 21-22, 2004 , NIH OBA held a safety symposium in conjunction with the meeting of the Recombinant DNA Advisory Committee (RAC). The symposium addressed safety considerations for recombinant DNA research with pathogenic viruses, focusing on containment issues for research with such viruses as 1918 influenza virus, highly pathogenic avian influenza viruses, and SARS Co-V. A major goal of the symposium was to develop a Points to Consider document and other resources to assist IBCs in conducting risk assessments and determining appropriate containment.

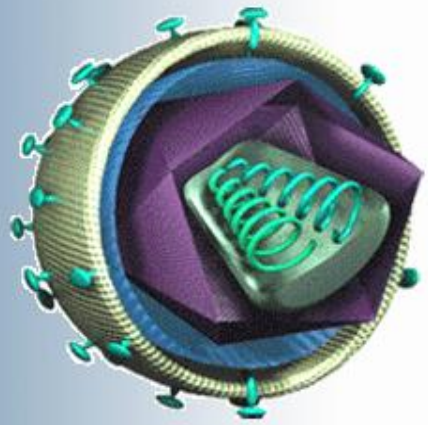
Agenda and Participant list

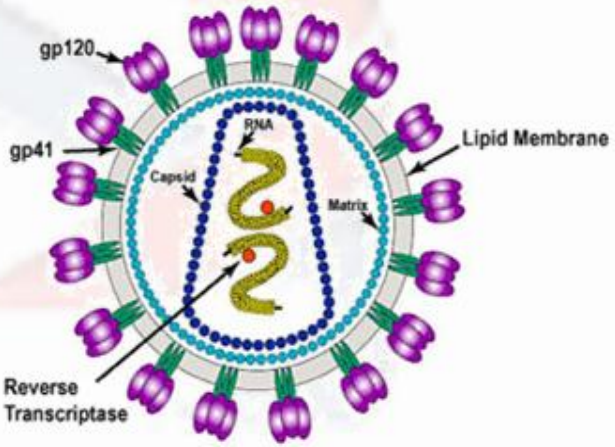
Webcast and slides

Resources

FAQs

Biosafety Considerations for Research with Lentiviral Vectors





At the March 15, 2006 RAC meeting, the RAC discussed the conduct of risk assessments and determination of containment for lentiviral vector research. The *NIH Guidelines for Research Involving Recombinant DNA Molecules* do not directly address the use of lentiviral vectors. To provide additional guidance to Institutional Biosafety Committees and investigators, OBA organized a working group of RAC and *ad hoc* reviewers with virology and biosafety expertise to develop some general criteria to be considered when conducting risk assessments for research involving lentiviral vectors.

NIH OBA Information Resources Pertinent to Human Gene Transfer Research

- GeMCRIS
- Guidance on Informed Consent for Gene Transfer Research
- HGT Research Participant Brochure

Information Resources from the NIH Office of Biotechnology Activities

Genetic Modification Clinical Research Information System (GeMCRIS)

A public database of human gene transfer trials registered with the National Institutes of Health

GeMCRIS

Genetic Modification Clinical Research Information System
Version 2.0

Home | Search | User Help

Support

- Feedback
- Frequently Asked Questions
- Contact Us
- Browser Requirements

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)

Site Map

Publicly Available Protocol Information

- Protocol title
- Study phase
- Clinical indication(s)
- Investigator(s)
- Clinical trial site(s)
- Scientific abstract
- Investigational strategy
- Vector
- Transgene
- Route of administration
- Non-technical abstract

Accessing GeMCRIS:

Connect to:

http://www.gemcris.od.nih.gov/

Information Resources from the NIH Office of Biotechnology Activities

NIH Guidance on Informed Consent for Gene Transfer Research

A new resource for investigators, IRBs, IBCs, potential research participants, and others concerned with informed consent in gene transfer trials

Informed Consent Guidance for Gene Transfer Research

Impetus

- **RAC review of informed consent documents revealed that investigators were having difficulty conveying important concepts pertinent to gene transfer research and to human subjects research more generally**
  - **inappropriately positive description of benefits**
  - **therapeutic misconception**
  - **presumptive use of the first person pronoun (“I understand that...”)**

National Institutes of Health
Office of Biotechnology Activities

NIH Guidance on Informed Consent For Gene Transfer Research

Appendix M-III-A-2
Comprehensibility

NIH GUIDELINES: "How will the major points covered in Appendix M-II, Description of Proposal, be disclosed to potential participants and/or their parents or guardians in a language that is understandable to them?"

DISCUSSION

Gene transfer research concepts are often difficult for potential participants to understand. Thus, particular care should be given to convey these concepts in the consent form in a readable and understandable manner. Readability and understandability are not synonymous; it is possible to make use of computerized readability scales and still have a consent form that is difficult to understand. Sometimes, reducing reading level without providing additional

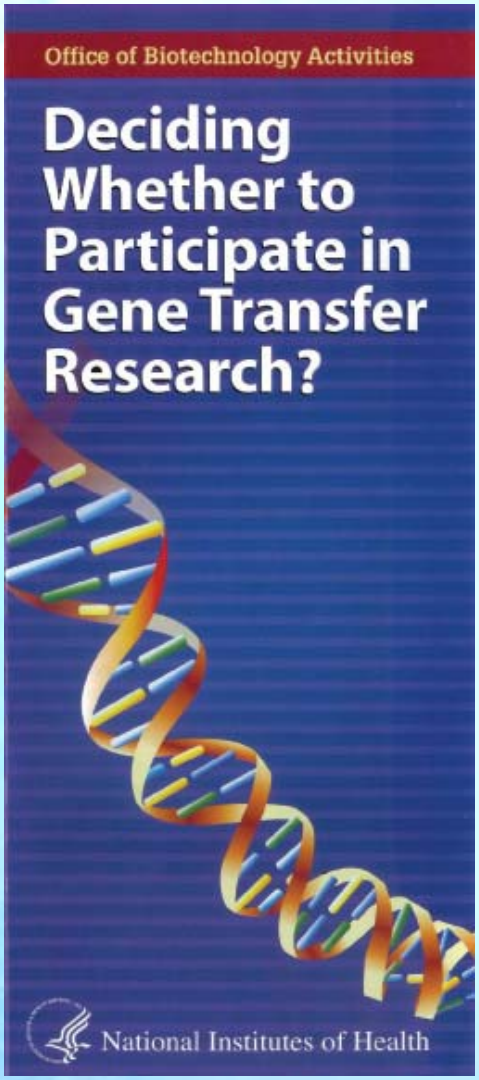
TOOLS & BACKGROUND MATERIALS

- Simplification Guide to Medical Terms
- Dartmouth Informed Consent Evaluation Tool
- Written Assessment Tool
- Telephone Evaluation Plan
- FDA Guidance on Non-English Speaking Subjects

MAIN POINTS

- Investigators should be attentive to using language easily read and understood by potential participants.
- Various methods and tools exist to improve and assess comprehension of information.
- All verbal and written

Informational Brochure for Potential Participants in Gene Transfer Research



- **Helps potential participants understand fundamental concepts in gene transfer research**
- **Suggests questions participants should pose to their physicians and to research staff in order to make a fully informed decision about participation**

NIH Office of Biotechnology Activities

Does your research involve recombinant DNA?

Then you should know about the:

NIH Office of Biotechnology Activities

OBA promotes science, safety and ethics in biotechnology through advancement of knowledge, enhancement of public understanding, and development of sound public policies. A core responsibility of OBA is to ensure that research involving recombinant DNA is conducted in keeping with the *NIH Guidelines for Research Involving Recombinant DNA Molecules*.

NIH Guidelines for Research Involving Recombinant DNA Molecules

The *NIH Guidelines* detail procedures and practices for the containment and safe conduct of various forms of recombinant DNA research, including research involving genetically modified plants and animals, and human gene transfer. A requirement of the *NIH Guidelines* is that an Institutional Biosafety Committee must review and approve all research subject to the *NIH Guidelines*.

Institutional Biosafety Committee

Institutional Biosafety Committees (IBCs) provide local review and oversight of nearly all forms of research utilizing recombinant DNA. They ensure that recombinant DNA research conducted at or sponsored by the institution is in compliance with the *NIH Guidelines*.

Have your projects been registered with the IBC?

IBC CONTACT _____

Visit OBA on the web: http://www4.od.nih.gov/oba or write to oba@od.nih.gov

NIH OBA Training, Education, and Information Resources

- **Staff and information resources available to help ensure IBCs, their institutions, and investigators are compliant with the *NIH Guidelines***
- **Scientific and medical staff available to answer queries**
  - **Interpretation of *NIH Guidelines***
  - **Containment**
  - **Exemptions**
  - **Risk group classification**

For Updates on All OBA Initiatives

- **Subscribe to OBA_NEWS**
  - **Email to: listserv@list.nih.gov**
  - **In body of message:**
    - **subscribe OBA_NEWS**

Questions?