



Gene Therapy: Charting a Future Course

National Institutes of Health (NIH)
Office of Science Policy
Office of Biotechnology Activities



Building 31C, 6th Floor, Room 10
Bethesda, MD
April 12, 2013

8:00 AM **Welcome and Opening Remarks**

Amy Patterson, M.D., Office of Science Policy, NIH, Bethesda, MD
[Slide Presentation](#)

Co-Chairs Howard Federoff, M.D., Ph.D., Georgetown University Medical Center,
Washington, DC

Yuman Fong, M.D., Chair, NIH Recombinant DNA Advisory Committee
Memorial Sloan Kettering Cancer Center, New York, NY

Session I: Future Frontiers for Gene Therapy

8:20 AM **Gene Therapy for Cancer**

Nabil Ahmed, M.D., M.P.H., Baylor College of Medicine, Houston, TX
[Slide Presentation](#)
Daniel Serman, M.D., University of Pennsylvania Medical Center,
Philadelphia, PA – [Slide Presentation](#)

9:00 AM **Gene Therapy for Ocular Diseases**

Shannon Boye, Ph.D., University of Florida, Gainesville, FL
[Slide Presentation](#)
Luk Vandenberghe, Ph.D., Harvard Medical School, Boston, MA
[Slide Presentation](#)

9:40 AM **Gene Therapy for Rare Diseases**

Scott Harper, Ph.D., Ohio State University College of Medicine,
Columbus, OH – [Slide Presentation](#)
Satiro De Oliveira, M.D., University of California, Los Angeles, CA
[Slide Presentation](#)

10:20 AM **New Approaches in Gene Therapy**

Aravind Asokan, Ph.D., University of North Carolina, Chapel Hill, NC
[Slide Presentation](#)
Yasuhiro Ikeda, D.V.M., Ph.D., Mayo Clinic, Rochester, MN
[Slide Presentation](#)

11:00 AM BREAK

Session II: NIH Resources

Beyond R01 Grants: Other NIH Resources for Translational Research

- 11:15 AM Clinical and Translational Science Awards**
Daniel Rosenblum, M.D., Division of Clinical Investigation,
National Center for Advancing Translational Sciences (NCATS), NIH
[Slide Presentation](#)
- 11:30 AM Gene Therapy Resource Program and National Gene Vector Biorepository**
Cheryl McDonald, M.D., National Heart, Lung, and Blood Institute, NIH
[Slide Presentation](#)
- 11:50 AM Recombinant DNA Advisory Committee/Genetic Modification Clinical Research Information System**
Jacqueline Corrigan-Curay, J.D., M.D., Office of Biotechnology Activities,
NIH – [Slide Presentation](#)
- 12:05 PM Rare Diseases Clinical Research Network**
Rashmi Gopal-Srivastava, Ph.D., Office of Rare Diseases Research, NCATS,
NIH – [Slide Presentation](#)
- 12:20 PM Questions**
- 12:30 PM Lunch**
- 1:15 PM Gene Therapy: Successes, Challenges, and Future Opportunities**
Ronald Crystal, M.D., Weill Cornell Medical College, New York, NY
[Slide Presentation](#)

Session III: Role of Foundations

- 1:30 PM Moderator:**
Donald Kohn, M.D., University of California, Los Angeles, CA
- Foundation Fighting Blindness**
Stephen Rose, Ph.D. – [Slide Presentation](#)
- National Tay-Sachs & Allied Diseases Association, Inc.**
Susan R. Kahn, M.B.A.

Muscular Dystrophy Association

Paul Muhlrاد, Ph.D. – [Slide Presentation](#)

Batten Disease Support and Research Association

Margie Frazier, Ph.D.

Alliance for Cancer Gene Therapy

Margaret Cianci, M.B.A.

2:30 PM Break

Session IV: Future Avenues and Issues

2:40 PM Key Bioethical Issues Raised in the Review of Gene Therapy Protocols

Laurie Zoloth, Ph.D. – [Slide Presentation](#)

2:50 PM Panel Discussion I

Moderators:

Laurie Zoloth, Ph.D., Northwestern University, Evanston, IL

Lawrence Prograis, Jr., M.D., National Institute of Allergy and Infectious Diseases, NIH

1. What are the main ethical issues that arise in gene therapy, and how can NIH help the field address these concerns?

3:20 PM Panel Discussion II

Moderators:

Howard Federoff, M.D., Ph.D.

Yuman Fong, M.D.

Lawrence Prograis, Jr., M.D.

Panelists:

Abraham Scaria, Ph.D., Sanofi-Genzyme, Framingham, MA

Angela Shen, M.D., M.B.A., Novartis Pharmaceuticals Corp., Florham Park, N.J.

Ronald Crystal, M.D.

What are the key scientific and technological challenges that must be overcome to continue to move this field forward?

1. What recent developments have been pivotal in advancing the field in the past 5 years?
2. What are the next key challenges to face in order to continue to move gene therapy forward?
3. In addition to grant funding, what other tools or resources would help facilitate this research?

4:45 PM

Biosafety and Communication Issues

1. What biosafety concerns still exist, and how can NIH help the field address these concerns?
2. Are there issues with public-perception? If so, how should the field address them?

5:15 PM

Adjourn