

**National Institutes of Health  
Recombinant DNA Advisory Committee (RAC)  
Bethesda Marriott Hotel  
Congressional Ballroom  
Bethesda, Maryland  
December 6, 2001**

**Meeting Agenda\***

Thursday, December 6, 2001

- 8:00 AM      **Call to Order**  
Claudia Mickelson, Ph.D., RAC Chair
- Tab 2232      For Your Information
- Notice of Meeting
  - Conflict of Interest Statement
  - Email From Dr. Dropulich Regarding Protocol #0107-488
  - Letter From Dr. Henrik Rasmussen and Jeffrey Carey, GenVec,  
    Regarding Protocol #0107-487
  - Letter From Dennis Swanson, IRB Office, University of  
Pittsburgh, Regarding Protocol #9902-285 and Followup
  - Response From Theresa Colecchia, Associate General Counsel,  
University of Pittsburgh
- 8:05 AM      **Opening Remarks: Appreciation for Service to the DHHS**  
William F. Raub, Ph.D., Deputy Assistant Secretary for Science Policy, DHHS
- 8:15 AM      **Presentation of RAC Member Certificates**  
William F. Raub, Ph.D., Deputy Assistant Secretary for Science Policy, DHHS  
Lana Skirboll, Ph.D., Director, Office of Science Policy, NIH
- 8:45 AM      **Spotlight on the RAC: Recent Accomplishments and Future Directions**  
Amy P. Patterson, M.D., Director, Office of Biotechnology Activities, NIH
- Tab 2231      Final Action for Serious Adverse Event Reporting
- 9:05 AM      **Perspectives From the RAC Chair**  
Claudia Mickelson, Ph.D.
- 9:25 AM      **Minutes of the September 6-7, 2001, RAC Meeting**  
RAC Reviewers:      Estuardo Aguilar-Cordova, Ph.D.  
                                 Sue Levi-Pearl
- Tab 2233      Minutes of the September 6-7, 2001, RAC Meeting

9:35 AM      **Proposed Response to Reported Appearance of Neoplasms After Vascular Growth Factor Gene Transfer**  
Jon Gordon, M.D., Ph.D., RAC Member

Tab 2235      Proposed Language for Inclusion to Letter to PI/Sponsor

10:15 AM      **Data Management Report**  
Jay Greenblatt, Ph.D., RAC Member

Tab 2236      Protocol List  
Protocols Not Requiring Full RAC Review  
Serious Adverse Event Summary Report  
Serious Adverse Event Comprehensive Report  
Amendments and Update Summary Table

10:30 AM      **BREAK**

10:45 AM      **Detection of Adeno-Associated Virus Vector Sequence in Research Participant Semen: Report and Analysis**

Tab 2237      Issue Brief and Background  
Additional Data and Analysis Submitted by PI  
Discussion Questions

RAC Working Group:      Estuardo Aguilar-Cordova, Ph.D.  
Xandra Breakefield, Ph.D.  
Theodore Friedmann, M.D.  
Jon Gordon, M.D., Ph.D.  
Nancy King, J.D.  
Louise Markert, M.D., Ph.D.  
Claudia Mickelson, Ph.D.

*Ad Hoc* Consultants:      Philip Johnson, M.D., Ohio State University  
Simon Hall, M.D., Mount Sinai Hospital  
Myron Cohen, M.D., University of North Carolina  
Jude Samulski, Ph.D., University of North Carolina  
Terence Flotte, M.D., University of Florida

10:50 AM      **Liver-Directed Gene Transfer of rAAV for Hemophilia B: Summary of Clinical Protocol and Data (Including Summary of Semen Vector Analysis) and Statement of Problem**  
Mark Kay, M.D., Ph.D., Stanford University

*Questions* (15 minutes)

- 11:35 AM     **Brief Explanation of Assay Used for Semen Vector Analysis**  
Deb Leonard, M.D., Ph.D., University of Pennsylvania
- Questions (15 minutes)*
- 12:05 PM     **Overview of Spermatogenesis**  
Simon Hall, M.D., Mount Sinai Hospital
- Questions (15 minutes)*
- 12:35 PM     **LUNCH**
- 1:35 PM     **Preclinical Studies of AAV-hFIX Vector Safety and Biodistribution**  
Kathy High, M.D., Children's Hospital of Philadelphia
- Questions (15 Minutes)*
- 2:20 PM     **Summary, Position Statement, and Clinical Trial Perspective**  
Elliot Grossbard, M.D., Avigen
- 2:40 PM     **FDA Procedures Regarding Potential Germ Line Transmission and Impact  
on Gene Therapy Clinical Trials**  
Stephanie Simek, Ph.D., Center for Biologics Evaluation and Research, FDA
- Questions (15 minutes)*
- 3:25 PM     **Public Comment**
- 3:45 PM     **RAC Working Group Analysis**
- 4:15 PM     **Discussion**
- 5:25 PM     **Closing Remarks**
- 5:30 PM     **Adjournment**

\* Meeting agenda as of 12/3/01 Please check the OBA Website for updates. Times are approximate and subject to change.