

**National Institutes of Health
Recombinant DNA Advisory Committee (RAC)
Building 45/Natcher Conference Center, Conference Room D
Bethesda, Maryland
September 6-7, 2001**

Draft Meeting Agenda*

Thursday, September 6, 2001

8:30 AM **Call to Order and Opening Remarks**
Claudia Mickelson, Ph.D., RAC Chair

Tab 2215 For Your Information
 - Notice of Meeting
 - Conflict of Interest Statement

8:40 AM **Minutes of the June 14-15, 2001, RAC Meeting**
RAC Reviewers: Jon Gordon, M.D., Ph.D.
 Sue Levi-Pearl

Tab 2216 Minutes of the June 14-15, 2001, RAC Meeting

8:45 AM **Clinical Protocol Updates**

**A Phase II Study of Autologous CD4-Zeta Gene-Modified T Cells in HIV-
Infected Patients With Undetectable Plasma Viremia on Combination
Antiretroviral Drug Therapy**
Dale Ando, M.D., RAC Member

Tab 2233 Protocol Summary

**A Multicenter, Double-Blind, Placebo-Controlled Phase II Study of
Aerosolized tgAAVCF in Cystic Fibrosis Patients With Mild Lung Disease**
Louise Markert, M.D., Ph.D., RAC Member
Nancy King, J.D., RAC Member

Tab 2234 Protocol Summary

* Meeting agenda as of 8/27/01. Please check the OBA Website for updates. Times are approximate and subject to change.

10:00 AM **BREAK**

10:15 AM **Discussion of Human Transfer Protocol #0107-485 entitled: Purging of Autologous Stem Cell Sources With *bcl-xs* Adenovirus for Women Undergoing High-Dose Chemotherapy for Stage IV Breast Carcinoma**

PI: Michael F. Clarke, M.D., University of Michigan, Ann Arbor

RAC Reviewers: Jon Gordon, M.D., Ph.D.
Theodore Friedmann, M.D.
Nancy King, J.D.

Ad hoc Reviewers: James Talmadge, Ph.D., University of Nebraska Medical Center
Charles Rudin, M.D., Ph.D., University of Chicago Medical Center
John Reed, M.D., Ph.D., Burnham Institute

Tab 2218 Protocol

Tab 2219 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews From Gordon, Friedmann, King, Talmadge, Rudin, and Reed
Sponsor's Response

11:30 AM **Discussion of Human Gene Transfer Protocol #0107-480 entitled: A Phase IIb, Randomized, Multicenter, Double-Blind Study of the Efficacy and Safety of *Trinam*[™] (EG004) in Stenosis Prevention at the Graft-Vein Anastomosis Site in Dialysis Patients**

PI: Valentin Fuster, M.D., Ph.D., Mount Sinai Medical Center

Sponsor: Ark Therapeutics, Ltd.

RAC Reviewers: Theodore Friedmann, M.D.
Dale Ando, M.D.

Ad hoc Reviewers: Michael Mann, M.D., University of California, San Francisco
Glen Nemerow, Ph.D., Scripps Research Institute

Tab 2220 Protocol

Tab 2221 OBA Summary

OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews From Friedmann, Ando, Mann,
and Nemerow
Sponsor's Response

12:45 PM **LUNCH**

1:45 PM **Discussion of Human Gene Transfer Protocol #0107-488 entitled: A Phase I, Open-Label Clinical Trial of the Safety and Tolerability of Single Escalating Doses of Autologous CD4 T Cells Transduced With VRX496 in HIV-Positive Subjects**

PI: Rob Roy MacGregor, M.D., University of Pennsylvania
 Medical Center

Sponsor: VIRxSYS Corporation

RAC Reviewers: Estuardo Aguilar-Cordova, Ph.D.

 Louise Markert, M.D., Ph.D.

 Nancy King, J.D.

Ad hoc Reviewers: John Zaia, M.D., Division of Virology, Beckman
 Research Institute and City of Hope

 Jiing-Kuan Yee, Ph.D., Division of Virology, Beckman
 Research Institute and City of Hope

 John M. Coffin, Ph.D., NCI and Tufts University School
 of Medicine

Tab 2222 Protocol

Tab 2223 OBA Summary
 OBA Letter to PI on RAC Public Review
 Outcome of Preliminary RAC Review
 Reviews From Aguilar-Cordova, Markert, King, Zaia,
 Yee, and Coffin
 Sponsor's Response

3:45 PM **BREAK**

4:00 PM **Data Management Report**
 Jay Greenblatt, Ph.D., RAC Member

Tab 2224 Protocol List
 Protocols Not Requiring Full RAC Review
 Serious Adverse Event Summary Report

Serious Adverse Event Comprehensive Report
Amendments and Update Summary Table
Correspondence Regarding Protocol #9902-285

4:30 PM **Communication of Issues Raised by Individual RAC Members Following the Preliminary RAC Review of Human Gene Transfer Protocols: Followup Discussion**
Nancy King, J.D., RAC Member

Tab 2217 Draft Language

5:00 PM **Proposed Response to Reported Appearance of Neoplasms After Vascular Growth Factor Gene Transfer**
Jon Gordon, M.D., Ph.D., RAC Member

Tab 2225 Slides

5:15 PM **Adjournment**

Friday, September 7, 2001

8:30 AM **Call to Order**
Claudia Mickelson, Ph.D., RAC Chair

8:35 AM **Discussion of Human Gene Transfer Protocol #0107-487 entitled: An Open-Label, Phase I, Single-Administration, Dose-Escalation study of AD_{ov}PEDF.11D (ADPEDF) in Neovascular Age-Related Macular Degeneration**

PI: Peter A. Campochiaro, M.D., Johns Hopkins University
School of Medicine

Sponsor: GenVec Inc.

RAC Reviewers: Xandra Breakefield, Ph.D.

 Claudia Mickelson, Ph.D.

 Louise Chow, Ph.D. (written review)

Ad hoc Reviewers: Joan Schwartz, Ph.D., National Institute of Neurological
Disorders and Stroke

 Martin Friedlander, M.D., Ph.D., Scripps Research Institute

 Marco Zarbin, M.D., Ph.D., University of Medicine and
Dentistry of New Jersey

Tab 2231 Protocol

Tab 2232 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews From Breakefield, Mickelson, Chow,
Schwartz, Friedlander, and Zarbin
Sponsor's Response

10:00 AM **Update on RAC Working Groups**
Xandra Breakefield, Ph.D., Chair, Vaccine Working Group
Claudia Mickelson, Ph.D., RAC Chair

Tab 2229 Roster
Charge to Committee

10:30 AM **BREAK**

10:45 AM **Amendments to the *NIH Guidelines***

Proposed Action on *E. coli* Risk Group Assessment
Eugene Rosenthal, Ph.D., OBA Biotechnology Advisor

Tab 2226 *Federal Register* Notice

**Proposed Action To Allow Modification of the Prescribed Number and
Expertise of RAC Members**
Amy Patterson, M.D., OBA Director

Tab 2227 *Federal Register* Notice

Final Action To Amend the Safety Information Reporting Requirements
Amy Patterson, M.D., OBA Director

11:15 AM **Update on IBC Policy Conference**
Allan Shipp, Director of Outreach, OBA

Tab 2228 Background Summary

11:30 AM **Donation of Ooplasm as a Treatment for Infertility and Its Implication
for the RAC**
Jon Gordon, M.D., Ph.D., RAC Member

Tab 2230 Slides

12:15 PM **Closing Remarks**

12:30 PM **Adjournment**