

**National Institutes of Health
Recombinant DNA Advisory Committee (RAC)
Building 31, Conference Room 10
Bethesda, Maryland
December 13 and 15, 2000**

Draft Meeting Agenda*

Wednesday, December 13, 2000

8 30 AM

Call to Order and Opening Remarks

Claudia Mickelson, Ph D , RAC Chair

Tab 2166

For Your Information

- Notice of Meeting
- Conflict of Interest Statement
- Draft Agenda for the 3rd Gene Transfer Clinical Research Safety Symposium Safety Considerations in Cardiovascular Gene Transfer Clinical Research

Correspondence

- Amendment to Protocol # 9508-118
- Letter from Michael Taggart, EBI, re withdrawal of Protocol #0010-425
- Letter from Alexander Kuta, Genzyme, re RAC recommendations to Protocol #0007-407
- Letter from Amy Patterson to Targeted Genetics re RAC recommendations to Protocol #0006-404
- Letter from Barry Polenz, Targeted Genetics re amendments to Protocol #0006-404

8 40 AM

Minutes of the September 25-26, 2000 Meeting

RAC Reviewers Estuardo Aguilar-Cordova, Ph D
Sue Levi-Pearl

Tab 2167

Minutes, September 25-26, 2000

8 45 AM

Review of "Timing Action" Recent Amendments to the NIH Guidelines Regarding Research Participant Enrollment and the Process of Protocol Submission and Review

Amy Patterson, M D , Director, Office of Biotechnology Activities
National Institutes of Health

Tab 2168

Federal Register Notice
Volume 65, Number 196, Pages 60328-60332

9 30 AM **Review of Replication Competent Retrovirus (RCR) Guidelines**
FDA

Tab 2169 Supplemental Guidance on Testing for RCR
Federal Register Notice
Volume 65, Number 202, Pages 62364-62365

10 30 AM **BREAK**

10 45 AM **Retroviral Vector Targeting**
Stephen J Russell, M D , Ph D
Director, Molecular Medicine Program, Mayo Clinic

Tab 2170 Review Articles

11 30 AM **LUNCH**

12 30 PM **Discussion of Human Gene Transfer Protocol #0010-417** entitled
*Tumor Site Specific Phase I/II Evaluation of Safety and Efficacy of
Hepatic Arterial Infusion of a Matrix-Targeted Retroviral Vector Bearing
a Dominant Negative Cyclin G1 (dnG1) Construct as Treatment for
Colorectal Carcinoma Metastatic to Liver*

PI Heinz-Josef Lenz, M D , University of Southern
California

Sponsor Erlinda Gordon, M D , Gene Therapy Labs

RAC Reviewers Xandra O Breakefield, Ph D

Dale G Ando, M D

Nancy M P King, J D

Ad hoc Reviewer Stephen J Russell, M D , Ph D , Mayo Clinic

Tab 2171 Protocol

Tab 2172 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews from Breakefield, Ando, King, Russell
Sponsor's Response

1 30 PM **Discussion on Human Gene Transfer Protocol #0010-423** entitled
Laminin 5 Beta 3 Gene Therapy for Junctional Epidermolysis Bullosa

PI Alexa B Kimball, M D , Stanford University

Sponsor N/A

	RAC Reviewers	Louise M Markert, M D Claudia A Mickelson, Ph D Ruth Macklin, Ph D
	Tab 2173	Protocol
	Tab 2174	OBA Summary OBA Letter to PI on RAC Public Review Outcome of Preliminary RAC Review Reviews from Markert, Mickelson, Macklin Sponsor's Response
2 30 PM	BREAK	
2 45 PM	Data Management Jay Greenblatt, Ph D , RAC Member	
	Tab 2175	Protocol List Protocols Not Requiring Full RAC Review Serious Adverse Event Summary Report Serious Adverse Event Comprehensive Report Amendments and Update Summary Table
3 45 PM	Development of the Gene Transfer Clinical Research Information System Overview James Foss, M B A , Project Manager, Stellar Systems	
	Tab 2176	Information System Presentation
4 15 PM	Scope and Applicability of the Guidelines Claudia Mickelson, Ph D	
	Tab 2177	
4 45 PM	End of Session	

Friday, December 15, 2000

8 30 AM	Opening Remarks Claudia Mickelson, Ph D
8 40 AM	Discussion of Human Gene Transfer Protocol #0009-411 entitled <i>Restenosis Gene Therapy Trial - Phase I Study (REGENT I)</i>

PI Richard E Kuntz, M D , M Sc , Beth Israel
Deaconess Medical Center and Harvard Medical
School

Sponsor North America LLC

RAC Reviewers Ted Friedmann, M D
Jon W Gordon, M D , Ph D
Sue L Levi-Pearl

Ad hoc Reviewer Edith Tzeng, M D , University of Pittsburgh

Tab 2178 Protocol

Tab 2179 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC REVIEW
Reviews from Friedmann, Gordon, Levi-Pearl, Tzeng
Sponsor's Response

9 40 AM

FDA Perspective on the Development of an Adenoviral Standard
Stephane Simek, Ph D
Center for Biologics Evaluation and Research, FDA

Working Toward an Adenoviral Vector Testing Standard
Beth Hutchins, Ph D
Director, Process Sciences, Canji, Inc

Tab 2180 Article "*Working Toward an Adenoviral Vector Testing
Standard*" by Beth Hutchins, et al

10 40 AM

BREAK

11 00 AM

Discussion of Human Gene Transfer Protocol #0005-398 entitled *A
Phase I Study of a Tropism Modified Adenovirus Vector for Intraperitoneal
Delivery of Therapeutic Genes in Ovarian and Extraovarian Cancer
Patients*

PI Mack N Barnes, M D , University of Alabama at
Birmingham

Sponsor N/A

RAC Reviewers Jon W Gordon, M D , Ph D
C Estuardo Aguilar-Cordova, Ph D
Ruth Macklin, Ph D

Ad hoc Reviewer Glen R Nemerow, Ph D , Scripps Research Institute

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Tab 2181 Protocol

Tab 2182 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews from Gordon, Aguilar-Cordova, Macklin
Sponsor's Response

12 00 PM

LUNCH

1 00 PM

Discussion of Human Gene Transfer Protocol #0010-419 entitled
*Intratumoral Injections of a Replication-Incompetent Adenoviral Vector
Encoding a Factor VII Immunoconjugate to Induce a Cytolytic Immune
Response Against Melanoma Tumors A Pilot Trial*

PI Albert Deisseroth, M D , Ph D , Yale University
Sponsor N/A
RAC Reviewers Louise T Chow, Ph D
Dale G Ando, M D
Nancy M P King, J D
Ad hoc Reviewer Jeffrey L Platt, M D , Mayo Clinic

Tab 2183 Protocol

Tab 2184 OBA Summary
OBA Letter to PI on RAC Public Review
Outcome of Preliminary RAC Review
Reviews from Chow, Ando, King
Sponsor's Response

2 00 PM

Discussion of Human Gene Transfer Protocol #0010-426 entitled *A
Phase I Study of Intratumoral Injections of OCaP1 for Metastatic or Locally
Recurrent Prostate Cancer, Part1 Dose Finding, Part 2 Index Lesion
Escalation*

PI Thomas A Gardner, M D , Indiana University
Sponsor DirectGene, Inc
RAC Reviewers Xandra O Breakefield, Ph D
Louise T Chow, Ph D
Sue L Levi-Pearl
Ad hoc Reviewer Glen R Nemerow, Ph D , Scripps Research Institute

Tab 2185 Protocol

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	Tab 2186	OBA Summary OBA Letter to PI on RAC Public Review Outcome of Preliminary RAC Review Reviews from Breakefield, Chow, Levi-Pearl Sponsor's Response
3 00 PM	BREAK	
3 15 PM	Proposed Action to Amend the NIH Guideline Requirements for Serious Adverse Event Reporting (SAER)	
	Tab 2187	
4 00 PM	Public Comment on Proposed SAER	
5 00PM	Adjournment Claudia Mickelson, Ph D	

* Draft meeting agenda as of 11/29/00 Please check the OBA Website for updates