

NATIONAL INSTITUTES OF HEALTH (NIH)  
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)

136<sup>th</sup> MEETING

6701 ROCKLEDGE DRIVE  
CONFERENCE ROOM 9100

DECEMBER 4-5, 2013

AGENDA

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*Wednesday, December 4, 2013*

12:30 PM            **Call to Order and Opening Remarks**

**Donald Kohn, M.D., RAC Chair**

Tab 2768        For Your Information  
                  Notice of Meeting  
                  Conflict of Interest Guidance

12:35 PM            **Minutes**

Tab 2769        Minutes of the September 11-12, 2013, RAC Meeting

RAC Reviewers:    Tianxi Cai, Sc.D.  
                          Angelica Hardison, M.B.A.

12:40 PM            **Discussion on Human Gene Transfer Protocol #1310-1260 titled: *A Three-Part, Multicenter, Open Label, Single Dose Study to Assess the Safety, Tolerability, and Efficacy of Intralabyrinthine (IL) Delivered CGF166 in Patients with Severe Hearing Loss***

PI:            Hinrich Staecker, M.D., University of Kansas Medical Center,  
                  Kansas City, KS

Sponsor:        Novartis Pharmaceuticals Corporation  
                          [Slide Presentation](#)

RAC Reviewers:    Rebecca Dresser, J.D.  
                          Walter Koch, Ph.D.  
                          David Ornelles, Ph.D.

*(continued) Wednesday, December 4, 2013*

Ad hoc Reviewer: Konstantina Stankovic, M.D., Ph.D., Harvard Medical School, Boston, MA

Tab 2770 Protocol

Tab 2771 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and Public Discussion  
Reviews from Drs. Koch, Ornelles, Stankovic and Professor Dresser  
PI's Response

2:15 PM **Public Comments**

2:20 PM **Discussion on Human Gene Transfer Protocol #1310-1262 titled: *A Phase I Study of Ad-RTS-hIL-12, an Adenoviral Vector Engineered to Express hIL-12, in Combination with Veledimex, an Oral Activator Ligand, in Subjects with Recurrent or Progressive Glioblastoma or Grade III Malignant Glioma***

PI: Antonio M. Omuro, M.D., Memorial Sloan-Kettering Cancer Center, New York, NY

Sponsor: Ziopharm Oncology, Inc.  
[Slide Presentation](#)

RAC Reviewers: William Curry, M.D.  
Joseph Pilewski, M.D.  
Laurie Zoloth, Ph.D.

Tab 2772 Protocol

Tab 2773 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and Public Discussion  
Reviews from Drs. Curry, Pilewski and Zoloth  
PI's Response

3:45 PM **Public Comments**

3:50 PM **Break**

*(continued) Wednesday, December 4, 2013*

- 4:00 PM                    **Discussion on Human Gene Transfer Protocol #1310-1253: Phase I/II Study of the Treatment of Metastatic Cancer that Expresses MAGE-A3 Using Lymphodepleting Conditioning Followed by Infusion of HLA-DP0401/0402 Restricted Anti-MAGE-A3 TCR-Gene Engineered Lymphocytes and Aldesleukin**
- PI:        Steven A. Rosenberg, M.D., Ph.D., National Cancer Institute, NIH, Bethesda, MD  
[Slide Presentation](#)
- RAC Reviewers:    Rebecca Dresser, J.D.  
                          Donald Kohn, M.D.  
                          Michel Sadelain, M.D., Ph.D.
- Tab 2774                Protocol
- Tab 2775                OBA Summary  
                          OBA Letter to PI on In-Depth RAC Review and Public Discussion  
                          Reviews from Drs. Kohn, Sadelain and Professor Dresser  
                          PI's Response
- 5:30 PM                **Public Comments**
- 5:40 PM                **Adjourn**

*Thursday, December 5, 2013*

- 8:30 AM                **Call to Order and Opening Remarks**
- 8:35 AM                **Gene Transfer Safety Assessment Board Report**  
[Slide Presentation](#)
- RAC Reviewers:    Michael Atkins, M.D.  
                          William Curry, M.D.  
                          Hans-Peter Kiem, M.D., Ph.D.  
                          Donald Kohn, M.D.  
                          Joseph Pilewski, M.D.  
                          Marshall Strome, M.D., M.S.  
                          Richard Whitley, M.D.

*(continued) Thursday, December 5, 2013*

Tab 2776            Responses to M-I-C-1  
                         Protocol List  
                         Protocols Not Selected for In-Depth Review and  
                         Public Discussion

8:45 AM

**Discussion of Human Gene Transfer Protocol # 1310-1263 titled: A Phase 2a Randomized, Multi-Center, Double-Blind, Placebo-Controlled, Sequential Dose Escalation Study to Assess the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of AG014 Administered Orally in Subjects with Moderate to Severe Active Ulcerative Colitis**

PI:        William Sandborn, M.D., University of California San Diego, La Jolla, CA

Sponsor:        ActoGeniX N.V.  
                         [Slide Presentation](#)

RAC Reviewers:    Marcella Sarzotti-Kelsoe, Ph.D.  
                         Marshall Strome, M.D., M.S.  
                         Laurie Zoloth, Ph.D.

Ad hoc Reviewer:    Vijay Yajnik, M.D., Ph.D., Harvard Medical School,  
                         Boston, MA

Tab 2777            Protocol

Tab 2778            OBA Summary  
                         OBA Letter to PI on In-Depth RAC Review and  
                         Public Discussion  
                         Reviews from Drs. Sarzotti-Kelsoe, Strome, Yajnik  
                         and Zoloth  
                         PI's Response

10:30 AM

**Public Comments**

*(continued) Thursday, December 5, 2013*

10:45 AM            **Discussion on Human Gene Transfer Protocol #1310-1266: A Phase I, Open-Label Clinical Trial Evaluating the Safety, Tolerability and Immunogenicity of Intradermally Administered ID-LV305 in Patients with Locally Advanced or Metastatic Cancer Expressing NY-ESO-1**

PI:        Neeta Somaiah, M.D., University of Texas, MD Anderson Cancer Center, Houston, TX

Sponsor:        Immune Design, Corp.  
[Slide Presentation](#)

RAC Reviewers:    Norman Fost, M.D., M.P.H.  
                         Marie-Louise Hammarskjöld, M.D., Ph.D.  
                         Dawn Wooley, Ph.D.

Tab 2779            Protocol

Tab 2780            OBA Summary  
                         OBA Letters to PI on In-Depth RAC Review and  
                         Public Discussion  
                         Reviews from Drs. Fost, Hammarskjöld and  
                         Wooley  
                         PI's Response

12:35 PM            **Public Comments**

12:45 PM            **Adjourn**