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Challenges in Advancing the Field of Gene Therapy: A Critical Review of the Science, Medicine and Regulation – ASGT Stakeholders Meeting

April 8, 2005

7:30 am – 8:00 am Continental Breakfast
8:00 am – 9:45 am

Session VI: Real Stories

Co-Chairs: Richard C. Mulligan, PhD and Cynthia Dunbar, MD
- Kenneth H. Fishbeck, MD – Muscular Dystrophy
- Carl H. June, MD – HIV and Lentivirus
- Douglas J. Jolly, PhD – Retroviral Factor VIII
- Ronald G. Crystal, MD – Gene Therapy for Batten Disease
- Panel Discussion

Break

10:00 am – 12 noon

Session VII: FDA/NIH Perspective

Co-Chairs: Stephanie Stinek, PhD and Kenneth Cornetta, MD
- Daniel Rosenblum, MD – Gene Transfer Studies: Clinical and Preclinical Issues of Regulatory Concern
- Andrew Byrne, PhD – Common Challenges in the Development of Gene Therapy Products
- Kenneth Cornetta, MD – Challenges to Gene Therapy: The IU NGVL Experience
- Additional Panelists for Discussion:
  - Monika C. McIntyre, PhD
  - Amy Patterson, MD
  - Mercedes Serubian, MS, DABT

1:00 pm – 3:00 pm

Session VIII: Final Discussions – Initiating the Process of “Consensus Thinking”: What Are the Directions Forward?

Co-Chairs/Moderators: Katherine A. High, MD and Daniel R. Salomon, MD

3:00 pm Adjourn

April 7-8, 2005 • Crystal Gateway Marriott • Salon IV, First Floor

Organizing Committee

Co-Chairs: Katherine A. High, MD The Children’s Hospital of Philadelphia Howard Hughes Medical Institute Philadelphia, PA
Daniel R. Salomon, MD The Scripps Research Institute La Jolla, CA

Members:
Dale Ando, MD Sangamo BioSciences Richmond, CA
Kenneth Cornetta, MD Indiana University/NGVL Indianapolis, IN
Philip R. Johnson, MD The Children’s Hospital of Philadelphia Philadelphia, PA
Douglas J. Jolly, PhD Advategene, Inc. Encinitas, CA
Carl H. June, MD University of Pennsylvania Philadelphia, PA
Robert W. Martin Genzyme Corporation Framingham, MA
Andra Miller, PhD The Biologics Consulting Group Bethesda, MD
Glenn F. Pierce, MD, PhD Avigen Alameda, CA
Sonia Skarlatos, PhD National Heart Lung and Blood Institute/NIH Bethesda, MD

Support has also been provided by:

This meeting was developed with the active participation of the FDA and NIH.

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Baylor College of Medicine
Jean Bennett, MD, PhD
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Malcolm K. Brenner, MD PhD
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Kenneth Cornetta, MD
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Maritza C. McIntyre, PhD
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John J. Nemnautis, MD
Mary Crowley Medical Research Center
Arthur Nienhuis, MD
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Savio L. C. Woo, PhD
Mount Sinai School of Medicine

April 7, 2005
7:30 – 8:15 am
Continental Breakfast and Registration
8:15 – 8:45 am
Welcome/Overview of Meeting
  · Katherine A. High, MD
8:45 – 11:00 am
Session I: Vectorology
  Co-Chairs: Arthur Nienhuis, MD and Barrie J. Carter, PhD
  Speakers:
  · David A. Williams, MD – Retroviral/Lentiviral
  · Cynthia Dunbar, MD – Pathophysiology of adverse events of the X-linked SCID trial.
  · Modeling insertional mutagenesis in non-human primates
  · Philip D. Gregory, PhD – Gene Correction Work as Alternative Therapeutic Modality
  · Panel Discussion
9:30 – 9:45 am
Break
Session I: Vectorology Continues
  · Terence R. Flotte, MD – AAV
  · Arthur L. Beaudet, MD – Adenovirus
  · Joseph C. Glorioso, PhD – Herpesvirus Vectors
  · Panel Discussion
11:00 am – 12:30 pm
Session II: Immunology of Gene Therapy
  Co-Chairs:
  · Savio L. C. Woo, PhD and Michel Sadelain, MD, PhD
  Speakers:
  · Daniel R. Salomon, PhD – Innate Immunity and the Paradox of Using Viral Pathogen-Based Vectors
  · Hildegund C. J. Erl, MD – Adaptive Immunity to Viral Vectors and to Transgene Products
  · Philip R. Johnson, MD – HIV Vaccine Trial
  · Panel Discussion
12:30 pm – 1:30 pm
Lunch Break
1:30 pm – 4:00 pm
Session III: Vaccines – Cancer and Other Diseases
  Co-Chairs: Hildegund C. J. Erl, MD and Dale Ando, MD
  Speakers:
  · Malcolm Brenner, MD, PhD – Retinoblastoma or Prostate or Both
  · Karin Jeos, PhD – GVAX Experience
  · John J. Nemnautis, MD – Trials with Adenovirus as a Vaccine
  · Stephen J. Russell, MD, PhD – Oncolytic RNA Viruses
  · Panel Discussion
3:30 pm – 3:45 pm
Break
3:45 pm – 5:00 pm
Session IV: Real Stories
  Co-Chairs: Samuel C. Wadsworth, PhD and Glenn F. Pierce, MD, PhD
  · Jean Bennett, MD, PhD – Pre-Clinical Studies for Lebers Congenital Amaurosis. Timeline from Compelling Pre-Clinical Studies to Initiation of Clinical Trials
  · Katherine A. High, MD – NAV Mediated Trials for Hemophilia
  · Mark H. Tuszynski, MD, PhD – Neural Cell Therapy
  · Panel Discussion
5:00 pm – 6:00 pm
Session V: Real Stories – The Role of Disease Foundations in Moving Gene Transfer Forward
  Co-Chairs: Alan Kinniburgh, PhD and Sharon Hesterlee, PhD
  · Rachel Salzman, DVM – The Stop ALD Foundation
  · Mark Skinner – World Federation of Hemophilia
  · Panel Discussion
6:00 pm – 7:00 pm
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Center for Cellular and Molecular Therapeutics at The Children’s Hospital of Philadelphia

Support has also been provided by:

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Pittsburgh, PA

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Alameda, CA

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