

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

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

The ASGT Stakeholders' Meeting is being **jointly sponsored** with the:



Center for Cellular and Molecular Therapeutics at  
The Children's Hospital of Philadelphia

Support has also been provided by:



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

## Overview

The Stakeholders' Meeting was created to address the issues presently faced by the field of gene therapy in translating the promise of this approach to successful clinical trials. Issues include the engineering and production of gene delivery vectors, concerns regarding insertional mutagenesis, the immunology of both the vectors and therapeutic gene products, effective antigen presentation and vaccine development, sustained and physiological expression of therapeutic genes, delivery of therapy to specific tissue compartments and a whole array of federal regulatory, safety, compliance and clinical trial design challenges, as well as the substantial financial resources required for clinical gene transfer studies.

Our strategy is to bring together members of the gene therapy community from academia, biotechnology and pharma, along with major disease foundations and leaders from the NIH and FDA to critically review the issues and consider ways to move the field forward and specifically facilitate the initiation and successful outcomes of clinical trials. The process will begin with a series of brief lectures on specific problem areas with the emphasis on identifying the nature of the barriers and options for circumvention. We will then develop a series of Real Stories based on recounting and critically analyzing actual experiences in clinical trials over the last 10 years.



## 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

Joseph C. Glorioso, PhD  
University of Pittsburgh School of Medicine  
Pittsburgh, PA

Philip R. Johnson, MD  
The Children's Hospital of Philadelphia  
Philadelphia, PA

Douglas J. Jolly, PhD  
Advantagene, 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

David A. Williams, MD  
Cincinnati Children's Hospital Medical Center  
Cincinnati, OH

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

## *Faculty*

Dale Ando, MD Sangamo BioSciences, Inc.	Carl H. June, MD University of Pennsylvania
Arthur L. Beaudet, MD Baylor College of Medicine	Alan Kinniburgh, PhD National Hemophilia Foundation
Jean Bennett, MD, PhD University of Pennsylvania Scheie Eye Institute	Maritza C. McIntyre, PhD CBER/FDA
Malcolm K. Brenner, MD PhD Baylor College of Medicine	Richard C. Mulligan, PhD Children's Hospital Boston
Andrew Byrnes, PhD CBER/FDA	John J. Nemunaitis, MD Mary Crowley Medical Research Center
Barrie J. Carter, PhD Targeted Genetics Corporation	Arthur Nienhuis, MD St. Jude Children's Research Hospital
Kenneth Cornetta, MD Indiana University/NGVL	Amy Patterson, MD OBA/OD/NIH
Ronald G. Crystal, MD Weill Medical College of Cornell University	Glenn F. Pierce, MD, PhD Avigen
Cynthia Dunbar, MD NHLBI, NIH	Daniel Rosenblum, MD FDA/CBER/OCTGT/DCEPT
Hildegund CJ Ertl, MD University of Pennsylvania Wistar Institute	Stephen J. Russell, MD, PhD Mayo Clinic
Kenneth Fischbeck, MD NINDS/NIH	Michel Sadelain, MD, PhD Memorial Sloan-Kettering Cancer Center
Terence R. Flotte, MD University of Florida	Daniel R. Salomon, MD The Scripps Research Institute
Joseph C. Glorioso, PhD University of Pittsburgh School of Medicine	Rachel Salzman, DVM The Stop ALD Foundation
Philip D. Gregory, PhD Sangamo BioSciences, Inc.	Mercedes Serabian, MS, DABT CBER/FDA
Sharon Hesterlee, PhD Muscular Dystrophy Association	Stephanie Simek, PhD CBER/FDA
Katherine A. High, MD The Children's Hospital of Philadelphia Howard Hughes Medical Institute	Mark Skinner World Federation of Hemophilia
Philip R. Johnson, MD The Children's Hospital of Philadelphia	Mark H. Tuszynski, MD, PhD University of California San Diego
Douglas J. Jolly, PhD Advantagene, Incorporated	Samuel C. Wadsworth, PhD Genzyme Corp.
Karin Jooss, PhD Cell Genesys	David A. Williams, MD Cincinnati Children's Hospital Medical Center
	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. Ertl, MD – Adaptive Immunity to Viral Vectors and to Transgene Products
- Philip R. Johnson, MD – HIV Vaccine Trial
- Panel Discussion

12:30 p.m – 1:30 pm

Lunch Break

1:30 pm – 4:00 pm

Session III: Vaccines – Cancer and Other Diseases

Co-Chairs: Hildegund C. J. Ertl, MD and Dale Ando, MD

Speakers:

- Malcolm Brenner, MD, PhD – Retinoblastoma or Prostate or Both
- Karin Jooss, PhD – GVAX Experience
- John J. Nemunaitis, 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 – AAV 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

Reception

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. Fischbeck, 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

9:45 am – 10:00 am

Break

10:00 am – 12 noon

**Session VII: FDA/NIH Perspective**

Co-Chairs: *Stephanie Simek, PhD* and *Kenneth Cornetta, MD*

- *Daniel Rosenblum, MD* – Gene Transfer Studies: Clinical and Preclinical Issues of Regulatory Concern
- *Andrew Byrnes, 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:
  - *Maritza C. McIntyre, PhD*
  - *Amy Patterson, MD*
  - *Mercedes Serabian, MS, DABT*

12 noon – 1:00 pm

Lunch Break

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*

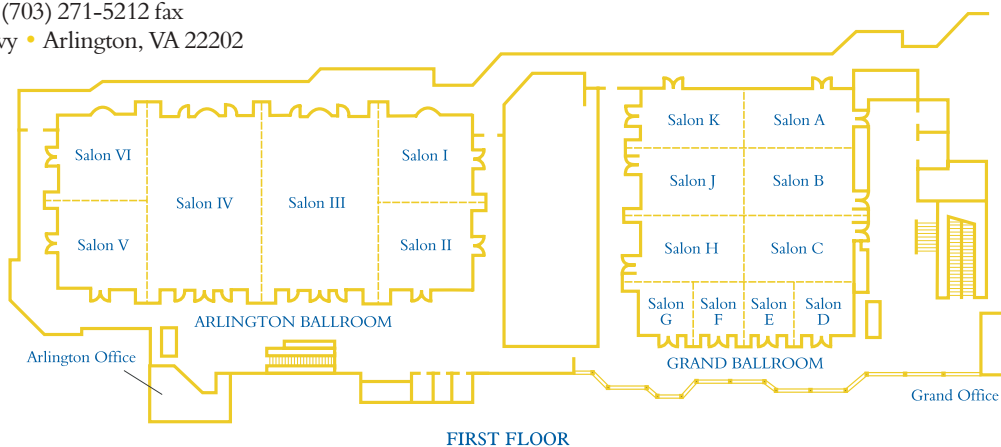
3:00 pm

Adjourn

**Crystal Gateway Marriott**

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