FRIDAY, February 22, 2008

8:00 am
Welcome and Introduction
Glenn F. Pierce, PhD, MD; Thierry VandenDriessche, PhD

Introduction to Hemophilia

8:15
Monitoring protein and transgene activities, use of thromboelastograms and thrombin generation assays
David Lillicrap, MD

Optimal Vector Production Systems

8:40
Overview of AAV vector production systems
J. Fraser Wright, PhD

8:55
AAV vector engineering and production in baculovirus systems
Janneke Meulenberg, PhD

9:10
Library-based approaches
Dirk Grimm, PhD

9:25
Efficient delivery of von Willebrand factor and ADAMTS13 via the SV40 in vitro packaging system
Chava Kimchi-Sarfaty, PhD

Preclinical Gene Therapy

9:40
AAV serotype tropism
Luk Vandenbergh, PhD

9:55
AAV-integration
Hiroyuki Nakai, MD, PhD

10:10
Break

10:30
FVIIa gene therapy
Paris Margaritis, PhD

10:45
Intra-articular gene therapy with AAV
Paul Monahan, MD

11:00
Helper-dependent adenoviral vectors for hemophilia
Arthur L. Beaudet, MD

11:15
Optimizing non-viral DNA vectors for liver directed gene transfer
Mark Kay, MD, PhD

11:30
Non-viral gene therapy for hemophilia
Michele Calos, PhD

11:45
Oral gene therapy for hemophilia A
Gonzalo Hortelano, PhD

12:00
Lunch
12:45
Improved viral and non-viral vectors for hemophilia B gene therapy
_Thierry VandenDriessche, PhD_

1:00
Hemophilia gene therapy using sleeping beauty transposons
_R. Scott McIvor, PhD_

1:15
Adult stem cell-mediated FVIII gene transfer
_David Lillicrap, MD_

1:30
Lentivirus expression of bioengineered FVIII
_Paul B. McCray, MD_

1:45
Hematopoietic stem cells
_Chrisopher Doering, PhD_

2:00
Celladon CUPID trial
_Thomas Cappola, MD, ScM_

2:15
Endothelial and platelet VWF and FVIII expression
_Qizhen Shi, MD, PhD_

2:30
Liver sinusoidal ECs
_Antonio Follenzi, MD, PhD_

2:45
Gene correction using zinc fingers
_Michael C. Holmes, PhD_

3:00
Integrate-defective lentiviral vectors and zinc finger nucleases for gene correction and targeted gene addition in human cells
_Angelo Lombardo, MSc_

3:15
Human hepatocytes repopulating liver
_Hongxiang Lan, PhD_

3:30
Break

**Gene Transfer Immunology**

3:50
Host-viral vector interactions: Overview
_Roland Herzog, PhD_

4:15
Immunomodulation for transgene-specific responses
_Carol Miao, PhD_

4:30
Innate immune responses to lentiviral vectors
_Brian Brown, PhD_

4:45
Vector immunology in the LPL muscle trial
_Federico Mingozzi, PhD_

5:00
Vector immunology: liver
_Katherine High, MD_

5:15
Protein immune responses to FVIII epitopes
_John Lollar, MD_
5:30
Autologous adoptive dendritic cell therapy for factor VIII tolerization
*Brendan Lee, MD, PhD*

6:00
Poster Reception

**Poster Presentations**

Hengjun Chao, MD  
*NHLBI/NHF R01 Grantee*  
*FVIII Immunity and Tolerance in AAV-Based Gene Transfer*

Gary E. Gilbert, MD  
*NHLBI/NHF R01 Grantee*  
*Conservative Enhancements of Factor VIII Functionality*

Yasuhiro Ikeda, PhD, DVM  
*NHF Career Development Awardee*  
*Generating Clinical Grade HIV-1 Vector Packaging Cell Lines for Hemophilia A Gene Therapy*

Junjiang Sun, MD  
*Judith Graham Pool Postdoctoral Research Fellow*  
*Direct Intra-Articular Delivery of Clotting Factor IX and AAV-Mediated Gene Therapy Contributes Protection against Blood-Induced Hemophilic Joint Pathology*

Alisa Wolberg, PhD  
*NHF Career Development Awardee*  
*Recombinant Factor VIIa Analog (V158D/E296V/M298Q-FVIIa, NN1731) Enhances Fibrin Formation, Structure and Stability in Hemophilic Plasma*
SATURDAY, February 23, 2008

Bioengineered Clotting Factors

8:00
Strategies to reduce ER stress responses
Randal J. Kaufman, PhD

8:15
Transgenic expression of milk-targeted FVIII
Steven Pipe, MD

8:30
Post-translational modifications of Vit K dependent clotting factors
Darrel Stafford, PhD

8:45
Novel approaches to improving VIIa activity
Alisa Wolberg, PhD

9:00
Increased potency and longer half life VIIa
Jesper Haaning, PhD

9:15
PEGylated/polysialylated FVIII and VWF: Longer acting FVIII by improved half-life
Peter Turecek, PhD

9:30
Factor Xa muteins
Rodney Camire, PhD

9:45
Factor VIII muteins
John E. Murphy, PhD

10:00
APC resistant FVIII
Andrew J. Gale, PhD

10:15
Break

Clinical Trials

10:35
Pegylated liposomes
Georg Lemm, MD, PhD

10:50
Clinical trial of a new recombinant Factor IX therapeutic agent
Edward D. Gomperts, MD

11:05
LPL deficiency clinical trials
Janneke Meulenberg, PhD

11:20
Muscular dystrophy using a mini dystrophin gene
K. Reed Clark, PhD

11:35
AAV for Parkinson’s disease clinical trials
Sam Wadsworth, PhD

11:50
AAV-FIX gene therapy
Amit Nathwani, MD, PhD

12:05
AAV-FIX gene therapy
Catherine Manno, MD
12:20  
Gene therapy for β-thalassemia  
Michel Sadelain, MD, PhD

12:35  
Concluding remarks

12:50  
Lunch

Glenn F. Pierce, PhD, MD; Thierry VandenDriessche, PhD
NINTH WORKSHOP ON NOVEL TECHNOLOGIES AND GENE TRANSFER FOR HEMOPHILIA
THE CHILDREN’S HOSPITAL OF PHILADELPHIA
FEBRUARY 22-23, 2008

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