

NINTH WORKSHOP ON NOVEL TECHNOLOGIES AND GENE TRANSFER FOR HEMOPHILIA
THE CHILDREN'S HOSPITAL OF PHILADELPHIA
FEBRUARY 22-23, 2008

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 Vandenberghe, 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

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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

Christopher 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

Antonia Follenzi, MD, PhD

2:45

Gene correction using zinc fingers

Michael C. Holmes, PhD

3:00

Integrase-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

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5:30
Autologous adoptive dendritic cell
therapy for factor VIII tolerization
Brendan Lee, MD, PhD

6:00
Poster Reception

NHF-Sponsored Research Fellows

7:30
Dinner
Penne Restaurant
The Inn at Penn
3600 Sansom Street

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*

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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

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12:20

Gene therapy for β -thalassemia
Michel Sadelain, MD, PhD

*Glenn F. Pierce, PhD, MD; Thierry
VandenDriessche, PhD*

12:35

Concluding remarks

12:50

Lunch

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